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UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

IN RE PTC THERAPEUTICS, INC. SECURITIES LITIGATION	Civil Action No. 16-1224(KM)(MAH) **CONSOLIDATED COMPLAINT and DEMAND FOR JURY TRIAL**

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Co-Lead Plaintiffs Boston Retirement System ("Boston") and Si Nguyen, Hong-Luu Nguyen, John Nguyen, and the Si Tan Nguyen Trust (the "Nguyen Family"), and additional plaintiff, Retail Wholesale Department Store Union Local 338 Retirement Fund ("Local 338") (collectively, "Plaintiffs"), individually and on behalf of all other persons and entities that, during the period from November 6, 2014 through February 23, 2016, inclusive (the "Class Period"), purchased or otherwise acquired the publicly traded common stock of PTC Therapeutics, Inc. ("PTC" or the "Company") and were damaged thereby (the "Class"), by their undersigned attorneys, allege in this Consolidated Class Action Complaint for Violation of the Federal Securities Laws (the "Complaint") the following upon knowledge with respect to their own acts, and upon information and belief.

Plaintiffs' information and belief concerning matters other than themselves and their own acts are based upon facts obtained through an investigation conducted by their counsel, which included, *inter alia*: (a) review and analysis of relevant filings made by PTC with the United States Securities and Exchange Commission (the "SEC"); (b) review and analysis of PTC's public documents, conference calls and press releases; (c) review and analysis of securities analysts' reports and advisories concerning the Company; (d) data and other information concerning PTC securities and the regulations under which PTC operates; (e) other publicly available information concerning the Company and the Individual Defendants; (f) an investigation conducted by and through Plaintiffs' attorneys and their investigators; and (g) consultation of an industry consulting expert in submission and approval of New Drug Applications (individually a "NDA") to the U.S. Food and Drug Administration ("FDA").

Plaintiffs believe that further substantial evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery.

NATURE OF THE ACTION

1. This is a federal securities class action brought by Plaintiffs on behalf of the Class seeking to recover compensable damages caused by Defendants' violations of federal securities laws, and pursue remedies under the Securities Exchange Act of 1934 (the "Exchange Act").

2. This action arises out of Defendants' misrepresentations and fraudulent course of conduct concerning PTC's development and implementation of clinical trials, and its subsequent submission to the FDA of a NDA for Translarna, the Company's blockbuster drug candidate developed to treat an extremely rare genetic disorder called nonsense-mutation Duchenne Muscular Dystrophy ("nmDMD").[1]

3. Unbeknownst to investors, these clinical trials were riddled with problems and improprieties, including cherry-picked data samples, failure to satisfy self-determined pre-specified success criteria, and post-hoc manipulation of data that cannot be used to obtain FDA approval. Moreover, both despite and as a result of these problems and improprieties, PTC's clinical trials failed to show a statistically significant treatment effect in young children stricken with nmDMD.

4. Unsurprisingly, but unknown to investors at the time, the NDA was summarily rejected by the FDA due to a series of facially obvious deficiencies. Such a summary rejection is communicated by the FDA through a Refuse-to-File ("RTF") letter, meaning that due to glaring deficiencies that are not a matter of interpretation, the FDA will not undertake even the standard initial step of filing a company's NDA.

[1] Nonsense-mutation DMD affects approximately 2,000 boys and young men in the U.S., and 2,500 boys and young men in Europe.

5. When the market finally learned about the true state of Translarna, through the RTF, the market reacted immediately, negatively, and severely, sending PTC's share price down by over ***$17.00 per share***, a one day loss of ***60%*** of its market cap.

A. Development of Translarna was a Bet-the-Company Move and Defendants Desperately Needed FDA Approval

6. While PTC had other drugs in preliminary development, Translarna was its first and only product for which the Company sought FDA approval during the Class Period. And analysts and investors fully recognized Translarna's importance to the Company's financial success. As one industry analyst put it, "***PTC is all about Translarna,*** the success or failure of Translarna is likely to define the Company over the next year or so . . . ."

7. PTC began developing Translarna in 2003. Following an initial round of clinical testing in 2011—resulting in highly negative findings—Translarna was rejected by the FDA. In the wake of this rejection, PTC benefitted substantially from the FDA's assistance in helping it design a "confirmatory" trial that would "wring out the risk" that had doomed the earlier trial and ensure swift FDA review and approval.

8. Specifically, Company executives met with the FDA to discuss the deficiencies the agency had identified, and how PTC could cure those deficiencies in subsequent clinical trials and NDAs. Indeed, it was based on these discussions with the FDA and the data from the 2011 trial that PTC ostensibly designed its subsequent clinical trial.

B. In Reality, PTC's Clinical Trials for Translarna Were Riddled with Problems and Improprieties

9. In reality, Defendants were unable, or unwilling, to follow the FDA's guidance, and failed to take full advantage of development benefits the FDA was willing to provide. Instead, they relied, almost exclusively, on a post-hoc analysis that allowed them to cherry-pick results and excluded a majority of the patient population they were aiming to treat. Not only was

this analysis unapproved by the FDA in pre-clinical trial meetings with PTC, it has been historically disfavored by the FDA because it invites biased data manipulation.

10. Indeed, when Defendants completed the 2015 "confirmatory" trial, the data showed that—as in the 2011 trial—Translarna did not meet PTC's own pre-specified criteria for success.

11. More distressingly, far from "confirming" Translarna's effectiveness, the 2015 clinical trial showed even worse results for the overall patient population than those tested in 2011. Likewise, the specific subgroup of boys afflicted with nmDMD for which 2015 trial was designed also failed to meet the threshold levels needed to show efficacy.

12. Finally, Defendants conducted improper post-hoc analyses that allowed them to cherry-pick certain beneficial data points. This impropriety, on its own, was enough to ensure that the FDA would not approve the Translarna NDA.

13. Indeed, FDA guidance makes clear that post-hoc analyses ***cannot be used to obtain FDA approval*** for a drug unless such analysis supports a clinical trial's pre-specified criteria for success. This rule is axiomatic—post-hoc analysis provides developers the opportunity to manipulate the interpretation trial data by cherry-picking only those results which most favorably demonstrate a drug's effectiveness.

C. Despite Knowing about Problems and Improprieties with the Translarna Trials, Defendants Nevertheless Told Investors that the Drug was On-Track for FDA Approval

14. Notwithstanding the known failings of the 2015 clinical trial, Defendants consistently touted throughout the Class Period Translarna's overall effectiveness at treating nmDMD. These misstatements created a false impression in the minds of the investing public and industry analysts that Translarna was destined for FDA approval. Indeed, by explicitly downplaying the known and material risk that the 2015 trial would fail, Defendants hid from the

investing public the substantial likelihood that the Company's NDA submission would be so facially insufficient as to cause the FDA to refuse to let PTC file it.

15. Moreover, throughout the Class Period, Defendants repeatedly emphasized to investors that they had learned important lessons from the failed 2011 trial which enabled them to "wring out the risk" and develop a foolproof design for the upcoming trial. Furthermore, Defendants also claimed that because they were able to discuss the "confirmatory" trial design and get crucial FDA feedback about the deficiencies in the earlier, rejected NDA, the upcoming clinical trial was "both well-designed and powered for success."

16. Most alarmingly, on October 15, 2015, Defendants announced the results of the "confirmatory" trial, actually exclaiming that it was a resounding success because "the totality of the data for Translarna demonstrated clinical benefit across primary and secondary endpoints" and "confirm[ed] clinical benefit of Translarna in [nmDMD]." Defendant Peltz even had the audacity to say that PTC was "proud to have confirmed the benefit of Translarna for the DMD patients."

17. These statements were false. Indeed, ***at the time they made these misrepresentations*** Defendants knew, but concealed, that the 2015 clinical trial had not only failed to meet its own pre-defined (and FDA approved) criteria for success, but yielded results ***considerably worse*** than those from the 2011 trial.

18. Moreover, in addition to defrauding the Company's investors, Defendants played fast and loose with the long-held hopes of parents of nmDMD children and their advocates for any drug that could have even a small effect on the progression of a debilitating genetic disorder that nearly always results in death during early adulthood. Indeed, throughout the Class Period Defendants freely sprinkled their misstatements about the clinical trials and Translarna's

supposed efficacy with suggestions that FDA approval would allow PTC to start "getting something to these kids."

19. Finally, because RTFs are rare, and indicate a rejection of a Company's NDA submission based on facial inadequacies identified during a fairly summary review of the NDA's contents, the deficiencies that caused the FDA to issue an RTF would have been glaring and fundamental. Indeed, FDA guidance states that:

> [A] RTF is based on ***omissions of clearly necessary information*** . . . or ***omissions or inadequacies so severe as to render the application incomplete on its face*** and where the ***omissions or inadequacies are so obvious***, at least once identified, and ***not a matter of interpretation or judgment*** about the meaning of the data submitted.

20. Therefore, because the overall 2015 trial of its bet-the-company drug failed to meet its own pre-specified (and FDA-approved) criteria for success, it is implausible that Defendants did not know the substantial likelihood that the FDA would refuse to review the Translarna NDA.

D. When the Market Learned About the Problems and Improprieties of the Translarna Clinic Trials, and the FDA's Refusal to Review the NDA, PTC's Share Price Plunged 60%

21. The misleading nature of Defendant's statements came to light on February 23, 2016, when PTC announced that the FDA had refused to file the Company's NDA. Defendants acknowledged publicly that the RTF was based primarily on the fact that "both the [2011] and [2015] studies ***had failed*** and therefore ***did not demonstrate substantial evidence of effectiveness***," and that "certain of the [C]ompany's adjustments to the [2015] study [were] ***post-hoc and therefore not supportive of effectiveness***," something Defendants had known all along.

22. On this news, the price of PTC shares ***fell over $17.00 per share*** on heavy trading, from its $28.26 closing price on February 22, 2016 to close at $10.84 on February 23, 2016. This one day ***60%*** drop represented a market cap loss of nearly ***$600 million***.

23. In response, industry analysts focused on PTC's dwindling credibility. One analyst from Cowen and Company said he felt misled by the Company's misstatements, explaining that "there is little transparency on FDA communications . . . to give investors comfort, and importantly, no additional transparency forthcoming despite eroded credibility."

JURISDICTION AND VENUE

24. This Court has jurisdiction over the subject matter of this action pursuant to 28 U.S.C. §§ 1331 and 1337 and Section 27 of the Exchange Act (15 U.S.C. § 78aa).

25. Venue is proper in this District pursuant to 28 U.S.C. § 1391(b), as the Company has its principal executive offices located in this District and a significant portion of its business, actions, and the subsequent damages, took place within this District.

26. In connection with the acts, conduct and other wrongs alleged in this Complaint, Defendants, directly or indirectly, used the means and instrumentalities of interstate commerce, including but not limited to, the United States mail, interstate telephone communications, and the facilities of the national securities exchanges.

PARTIES

27. Co-Lead Plaintiff Boston is a defined-benefit governmental pension plan headquartered in Boston, Massachusetts. Boston manages assets on behalf of beneficiaries associated with the City of Boston, including the Boston Redevelopment Authority, the Boston Housing Authority, the Boston Water and Sewer Commission, and the Boston Public Health Commission. As of December 31, 2015, Boston managed approximately $5.7 billion in net assets on behalf of more than 43,410 members and their beneficiaries. Boston purchased PTC

common stock during the Class Period, as set forth in the certification previously filed with the Court (*see* ECF No. 17-3),[2] and suffered damages as a result of the federal securities law violations alleged herein. By order dated November 14, 2016 (*see* ECF No. 44), this Court appointed Boston as Co-Lead Plaintiff in this action.

28. Co-Lead Plaintiff the Nguyen Family purchased PTC common stock during the Class Period, as set forth in the certification previously filed with the Court (*see* ECF No. 18-3),[3] and suffered damages as a result of the federal securities law violations alleged herein. By order dated November 14, 2016 (*see* ECF No. 44), this Court appointed the Nguyen Family as Co-Lead Plaintiff in this action.

29. Additional Plaintiff Local 338 purchased PTC common stock during the Class Period, as set forth in the certification previously filed with the Court (*see* ECF No. 16-4),[4] and suffered damages as a result of the federal securities law violations alleged herein. By order dated November 14, 2016 (*see* ECF No. 44), this Court approved of Local 338's listing as an Additional Plaintiff to represent the Class in this action.

30. Defendant PTC is a Delaware corporation with its principal executive offices located at 100 Corporate Court, South Plainfield, New Jersey 07080. Incorporated in 1998, PTC is a biopharmaceutical company that focuses on the discovery, development and commercialization of orally administered therapies, which target several rare genetic disorders, including but not limited to nonsense-mutation Duchenne Muscular Dystrophy.

[2] Boston's previously filed certification is incorporated herein by reference.

[3] The Nguyen Family's previously filed certification is incorporated herein by reference.

[4] Local 338's previously filed certification is incorporated herein by reference.

31. Defendant Stuart Peltz, Ph.D. ("Peltz") is and was throughout the Class Period Chief Executive Officer ("CEO") and Executive Director of PTC. Prior to founding PTC in 1998, Peltz was a Professor in the Department of Molecular Genetics and Microbiology at the Robert Wood Johnson Medical School, Rutgers University. He received a Ph.D. from the McArdle Laboratory for Cancer Research at the University of Wisconsin. In addition to having published more than 100 publications in his area of expertise, Peltz has also received a number of scientific awards and was elected as a Fellow of the American Academy for the Advancement of Science in 2010. Dr. Peltz also serves on National Institutes of Health and American Cancer Society review committees.

32. Defendant Shane Kovacs ("Kovacs", and together with Peltz, the "Individual Defendants") was, at all relevant times, Chief Financial Officer ("CFO") of PTC. Prior to joining PTC, Kovacs served as Managing Director of Health Care Investment Banking at Credit Suisse, where he advised life sciences companies such as PTC on various financial transactions, including raising funds. Kovacs received a Bachelor's of Engineering in Chemical Engineering and a Bachelor's of Sciences from Queen's University in Kingston, Ontario in 1998, and subsequently received an MBA from the University of Western Ontario in 2002. Mr. Kovacs is also a CFA Charter holder.

33. PTC, Peltz, and Kovacs are referred to herein as "Defendants".

SUBSTANTIVE ALLEGATIONS

A. Peltz Founded PTC to Develop Drugs for Rare Genetic Disorders, and Developed Translarna to Treat Duchenne Muscular Dystrophy

34. PTC is a biopharmaceutical company focused on the discovery, development, and commercialization of orally administered therapies that operate through a process called post-

transcriptional control.[5] Defendant Peltz and others founded PTC in 1998 to develop therapies using post-transcriptional control to treat underserved disorders and diseases. PTC focuses its efforts primarily on the development and commercialization of treatments for orphan and ultra-orphan disorders that affect a very small percentage of the population.[6] But in order to make commercialization of treatments for such rare disorders profitable for the Company and its investors, such orphan disease treatments typically command some of highest prices in the pharmaceutical industry.

35. In 2003, the Company began developing Translarna, which theoretically uses post-transcriptional control to target and treat a specific genetic mutation called a nonsense mutation. Nonsense mutations prevent the production of full-length, functional proteins which, depending on the protein, can result in a variety of genetic disorders, including DMD, Cystic Fibrosis, and others. In theory, Translarna allows a cell to "read-through" the nonsense mutation to produce functional proteins, slowing the progression of the disorder.

36. While PTC does have other drug candidates under development, Translarna was by far PTC's most advanced product candidate throughout the Class Period, and was its first product for which the Company would seek regulatory approval in the U.S. and Europe. Translarna also represented PTC's only opportunity to generate any revenues for the foreseeable future, since other treatments the Company had under development were years from commercialization. Indeed, during the Class Period, securities analysts fully recognized

[5] Post-Transcriptional Control treats deficiencies caused by genetic mutations that prevent proper gene expression.

[6] An orphan designation is a status conferred on drugs intended for the safe and effective treatment of diseases that affect fewer than 200,000 people in the U.S. Ultra-orphan disorders, though not formally recognized by the FDA, are even more rare than orphan disorders—they generally have a prevalence in the overall population of 1 in 2,000 to 1 in 50,000.

Translarna's importance to the Company's financial success (or failure). As one securities analyst from Roth Capital Partners noted: "***PTC is all about Translarna, the success or failure of Translarna is likely to define the Company over the next year or so***. . . ."

37. Translarna is also unique because, according to the scientific theory underlying post-transcription control, it could be used to treat any disorder that is caused by a nonsense mutation. During the Class Period, PTC was most progressed in commercializing Translarna for treatment of nmDMD (although the Company was also investigating the use of Translarna for other disorders). Accordingly, success in the first Translarna clinical trials and subsequent FDA approval process for DMD were crucial because a successful trial and NDA would greatly influence the FDA's overall opinion on Translarna, including for other indications.

1. PTC Targeted nmDMD as the First Disorder to be Treated by Translarna

38. The first disorder PTC targeted for treatment with Translarna was a specific type of muscular dystrophy known as Duchenne muscular dystrophy ("DMD"). The disorder occurs predominantly in young boys who typically begin showing symptoms as early as two or three years old. Young children suffering from DMD generally experience a slow weakening and eventual wasting of muscle tissue. Muscle loss usually occurs first in the upper legs and pelvis followed by those of the upper arms. This results in difficulty walking and standing with most patients unable to walk by adolescence.

39. Catastrophic muscle loss in DMD patients is caused primarily by their body's inability to properly produce a protein called "dystrophin," which normally supports the structure of muscle cells. Without dystrophin, muscle cells break down as a result of normal muscle use; muscle function declines as fat or other fibrous cells take the place of damaged muscle cells. Beginning around the age of ten, respiratory function also weakens, requiring the children to use a respirator. The disease eventually affects the heart muscles, leading to heart failure, and death.

2. PTC Obtained Orphan Drug and Fast Track Designations from the FDA for Translarna

40. Because nmDMD afflicts only a very small patient population, PTC was able to benefit from the FDA's "orphan drug" designation throughout much of Translarna's development, including the clinical trials and data analyses.[7] This designation provided PTC with numerous development incentives (as a result of the Orphan Drug Act), including tax credits for qualified clinical testing and market exclusivity.

41. As developers of an orphan drug, PTC also received a grant through the FDA's Orphan Products Clinical Trial Grants Program,[8] funds clinical trials that a company must run to gain FDA approval. This assistance is particularly valuable to developers such as PTC, especially when one considers that a clinical trial typically costs $30-40 million to design and implement. Although the designation provides assistance for developers of drugs for orphan disorders, FDA guidance is emphatic that "[t]he granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies."

42. Similarly, PTC filed for and obtained "fast track" designation for Translarna. Fast track is another FDA program intended to facilitate the development, and expedite the review of drugs intended to treat serious conditions and unmet medical needs. PTC's fast track designation enabled the Company to take advantage of numerous benefits (in addition to those provided by

[7] PTC was also granted orphan drug designation for the treatment of nonsense mutation cystic fibrosis ("nmCF") and a disorder known as spinal muscular atrophy ("SMA").

[8] As discussed in further detail in Paragraph 41 below, PTC used a grant from this FDA program to help fund its Translarna Phase 2b trial for nmDMD that ultimately failed to show efficacy according to the Company's pre-specified criteria.

the orphan drug designation), including (among others): (1) more frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval; (2) more frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers; and (3) Rolling Review, which allows a drug company to submit a NDA for review as data analyses and other portions of the application come in (rather than waiting for every element of the NDA to be finished before the entire application can be reviewed). According to the FDA's guidance:

> Once a drug receives *Fast Track* designation, ***early and frequent communication*** between the FDA and a drug company ***is encouraged*** throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients

43. Even though this additional guidance is available to PTC and encouraged by the FDA, it was up to the Company to take advantage of the FDA's extra help. Indeed, as would become evident by the end of the Class Period, the FDA's ultimate rejection of PTC's second NDA for Translarna for nmDMD is evidence that the Company was ***not*** in close communication with the FDA, and had recklessly failed to take advantage of the valuable benefits afforded it by virtue of its orphan drug and "fast track" designations.

3. The FDA Requires Submission of a New Drug Application Demonstrating Substantial Evidence that the Treatment is Effective

44. Regardless of any of the benefits available to PTC by virtue of such designations, the Company was otherwise required to engage in the full standard process of drug approval by filing a NDA prior to marketing and selling its treatment in the United States. Specifically, a NDA must show "substantial evidence" that the drug is safe and effective at treating the condition it purports to treat. Indeed, all drugs currently marketed within the United States were, at some point, the subject of an approved NDA.

45. A properly submitted NDA provides the FDA with all pertinent information about the drug, including data and statistical analyses sufficient to determine whether: (1) the drug is safe and provides the benefits it purports to; (2) the benefits of the drug outweigh its risks; (3) the drug's proposed labeling is appropriate and what it should contain; and (4) the methods used in manufacturing the drug and the controls used to maintain the drug's quality are adequate to preserve the drug's identity, strength, quality, and purity. Although safety and efficacy are particular important in the FDA's assessment, each of these issues is independently critical to the agency's ultimate approval decision.

46. In order to meet these standards, drug developers typically subject a drug candidate to a series of clinical trials designed to accumulate the data required to submit a successful NDA. Phase 1 clinical trials typically evaluate an investigational drug's safety and dosage tolerance. Phase 2 clinical trials: (1) usually involve larger patient populations; (2) evaluate dosage tolerance and appropriate dosage; (3) identify possible short-term adverse effects and safety risks; and (4) provide a preliminary evaluation of the efficacy of the drug for specific indications.

47. Phase 2 clinical trials are sometimes conducted in multiple sub-phases denoted as Phase 2a and Phase 2b. Phase 2a trials are typically smaller and provide additional introductory information on drug safety and efficacy, and are insufficient to independently support a NDA. Phase 2b clinical trials are usually larger and may be for longer durations of time, and may support a NDA. More importantly, in most Phase 2b trials, including the Phase 2b trial for Translarna, an investigational drug's efficacy is measured against placebo.

48. Finally, Phase 3 clinical trials test for efficacy and safety in an even further expanded patient population. Phase 3 trials also usually involve comparison with placebo, and

are intended to establish the overall risk-benefit profile of the product and provide an adequate basis for physician labeling.

49. The FDA typically requires two successful clinical trials—which must be "adequate and well-controlled investigations"—in order to provide the FDA with "substantial evidence" that a drug is safe and effective. The FDA has generally emphasized the plural in "investigations," meaning that most drug approvals are based on the results of at least two pivotal trials.

50. FDA procedures make "submission" and "filing" distinct events. Specifically, a pharmaceutical company "submits" a NDA to the FDA.[9] After a preliminary review for facial sufficiency, the FDA "files" the application into a dossier, where it remains until further action is taken by regulators for a complete substantive review.

51. Thus, the act of "filing" a pharmaceutical company's submission is a decision by the FDA that the submission merits the resources required for substantive review. But if the FDA receives an application that is incomplete, improperly constructed, or otherwise facially inadequate, it issues a RTF letter informing the drug developer that the application will not receive further agency attention until the problems are corrected.

4. The FDA Refuses to File Only Those NDAs Which Are Facially Inadequate

52. According to the FDA:

> [i]n general, a RTF is based on ***omissions of clearly necessary information*** . . . or ***omissions or inadequacies so severe as to render the application incomplete on its face*** and where the ***omissions or inadequacies are so obvious***, at least once identified, and ***not a matter of interpretation or judgment*** about the meaning of the data submitted."

[9] In the case of PTC, the Company submitted its 2015 NDA for Translarna for nmDMD through a rolling review process while the Phase 3 DMD trial was still underway.

RTFs are not appropriate for issues related to the balancing of risks and benefits, magnitude of clinical effect, nuances of study design, or other complex issues and close judgments—these are matters assessed ***after*** filing, when FDA regulators are deciding whether to approve the NDA.

53. Because the FDA does not release information on the subject (and because only publicly traded companies that would experience a RTF as a material event are obligated to disclose their receipt), there is no way to determine from publicly available sources exactly how many submissions are rejected via RTF prior to substantive review. Nonetheless, all available resources indicate that, when it comes to applications for new molecular entities (the category into which PTC's Translarna application falls), RTFs are relatively rare—of more than 200 total applications submitted between January 2010 and February 2016, only 18 received RTFs, two of which concerned Translarna.

54. One factor in the relative rarity of RTFs is that modern drug development, especially for orphan drugs such as Translarna, is premised on the availability of frequent communication between the drug developer and the FDA, meaning that any concerns or issues that the FDA might express should be addressed prior to completion of the submission.

B. Translarna Failed its First Major Efficacy Trial

55. By May 2007, PTC had completed the Phase 1 and Phase 2a trials for Translarna and began designing the first major trial (a Phase 2b trial) intended to demonstrate that Translarna is effective in treating children with nmDMD. PTC began enrolling patients in the Phase 2b trial in February 2008. Ultimately, the trial included 174 nmDMD patients between the ages of 5 and 20 and, at the time, was the largest randomized, double-blind, placebo-controlled clinical trial for DMD in history. This trial was funded, in part, by federal funds in the form of a grant from the FDA Orphan Products Clinical Trials Grant Program. This constitutes further

evidence that the FDA was more than willing to help PTC in getting Translarna approved, if only PTC was willing to take advantage of the breadth of the FDA's expertise.

1. PTC Set Phase 2b Clinical Endpoints Intended to Measure Translarna's Efficacy

56. The Company's trial design was intended to show a slower decline in a Translarna-treated patient's ability to use his muscles, as measured by the distance a patient could walk over the course of 6 minutes.[10] This distance was to be measured at week one of the trial (also known as baseline) and then again at the end (week 48). Findings for the Translarna-treated population were then compared against the findings for those DMD patients taking placebo.

57. PTC believed that if Translarna was effective in slowing the degeneration of muscle, therefore those patients receiving treatment would experience a smaller decline in their 6-minute walk distance over a 48-week period, than those on placebo would.

58. Prior to beginning the Phase 2b trial, PTC pre-specified the criteria it would use to establish Translarna's effectiveness at treating nmDMD. They also set the goalpost—the drug would be deemed effective at treating nmDMD only if the distance walked by children taking Translarna exceeded that of patients on placebo by more than 30 meters (on average) as of week 48 of the trial. This would show that those patients' ability to walk declined less and would represent a clinically meaningful benefit for the purposes of the trial.

[10] This procedure is known as a 6-minute walk test ("6MWT"), and the resulting metric is called the 6-minute walk distance ("6MWD").

59. To rule out the probability of a false positive result, PTC also pre-specified that the results would have to achieve a benchmark p-value of 0.05 or less to be considered statistically significant and deemed demonstrative of Translarna's clinical effect.[11]

2. Although the Translarna Phase 2b Trial Failed to Meet Clinical Endpoints, PTC Lauded the Trial's "Success"

60. The Translarna Phase 2b study was completed in December 2009. Three months later, on March 3, 2010, the Company released the preliminary results of the Phase 2b trial. While the test results showed some modest improvements for those patients taking Translarna, the overall results fell short of the Company's own pre-specified criteria for clinically meaningful benefit. Just as importantly, those results also fell well short of the requisite statistical significance. Accordingly, the Phase 2b trial did ***not*** demonstrate that Translarna was effective at treating nmDMD.

61. PTC blamed the poor results and lack of statistical significance on "younger patients and patients with higher baseline 6-minute walk distances [that] are less likely to exhibit measurable declines in 6-minute walk distance over 48 weeks." The Company explained that these younger children, while suffering from DMD, were not far enough progressed in the disorder at their initial assessments to see a likely clinical effect during the course of the 48-week clinical trial (*i.e.*, their muscle function was unlikely to degrade enough to benefit from Translarna).

62. Despite these results, the Company publicly lauded in press releases, SEC filings, and during earnings calls with analysts that the trial was a success and demonstrated Translarna's

[11] The 0.05 level of statistical significance (also shown as p=0.05) is typical amongst clinical trials, and simply means that there is only a 5% likelihood that the outcome is the result of chance alone.

efficacy. The Company's enthusiasm was based on several improper retrospective, or ***post-hoc***, data analyses through which the Company was purportedly able to show a statistically significant benefit, albeit for only a small sub-segment of the original population PTC intended to treat in the Phase 2b trial.

63. Such post-hoc analyses cannot be used to obtain FDA approval for the drug (if it is not supportive of the trial's primary endpoint), because they provide developers the opportunity to manipulate the interpretation of the trial's data by cherry-picking only that which most favorably demonstrates the drug's effectiveness.

3. The FDA Refused to File PTC's NDA for "Conditional Approval" of Translarna

64. PTC submitted in March 2011 a NDA (the "Phase 2b NDA") based on the results of the Phase 2b trial, apparently hoping that the FDA would overlook the clinical trial's failure to meet its overall criteria by which the drug's effectiveness were to be measured. Understanding that the FDA would view the results as a failure, PTC as part of their NDA also submitted the post-hoc analyses that focused on an older, declining sub-segment of the trial population. PTC posited that this subgroup had shown a clinical benefit, further bolstering the Company's claim that data from the younger children whose disorder was less progressed were the cause of the negative Phase 2b results.

65. Not long after, the FDA sent the Company a RTF (the "Phase 2b RTF"), indicating that the NDA was ***facially deficient*** on the grounds that "the single placebo controlled Phase 2b clinical trial contained in the NDA ***did not achieve statistical significance in the pre-specified analysis***," something the Company had known all along.

66. In December 2011, the Company filed a formal dispute resolution request with the FDA regarding the RTF, but in January 2012, the FDA reaffirmed the appropriateness of its earlier decision.

C. PTC Engaged in Substantive Discussions with the FDA Following the Phase 2b RTF, Gaining Invaluable Insight into the Requisite Contents for a Successful NDA for a Drug to Treat nmDMD

67. In February 2012, after receiving notice that the FDA would not hear the Company's appeal, PTC executives engaged in a number of meetings and discussions with the FDA as to how to get Translarna approved. During these meetings, PTC engaged in discussions with the very regulators that would review the Company's submission, giving it the opportunity to find out exactly what the agency needed as "substantial evidence" of Translarna's efficacy.

68. PTC executives also received feedback about the deficiencies the FDA found in the Phase 2b NDA. These meetings provided PTC with the opportunity for feedback from the very agency that would adjudicate the acceptability of their Phase 3 trial design, proposed endpoints, and statistical analysis plan; this trial was also known as the "Ataluren [Translarna] Confirmatory Trial" or ACT DMD.

69. In designing the study and the statistical analysis plan, Defendants could minimize the likelihood of failure by tailoring the design to the results they had observed in the Phase 2b study, and by implementing the FDA's advice about the particular means by which the FDA would assess efficacy. For example, on August 12, 2013 during an earnings call with analysts, Defendant Peltz explained how the FDA's input helped shape the trial, noting: "[t]he design of the trial reflects the knowledge gained from our earlier study as well as the views expressed in discussions with the FDA. . . ." Defendants even continued to tell investors during the Class Period that the meetings with the FDA and the assessment of the Phase 2b data allowed them to "wring out the risk" from the Phase 3 trial.

70. Indeed, on several occasions during the Class Period, Defendants disclosed that some of those meetings addressed the Company's detailed statistical analysis plan that Defendants submitted to the FDA for its Phase 3 trial. For example, in response to a comment about the Company's discussions with the FDA about its Phase 3 statistical analysis plan, Defendant Peltz noted "[t]he pre-specified meta-analysis was in our statistical analysis plan, which we had discussions with with [sic] the FDA. . . . So they are well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure."

D. PTC Supposedly Designed and Began Enrollment of the "Confirmatory" Phase 3 Trial that was "Powered for Success" Based on Lessons Learned from the Failed 2B Trial and Specific FDA Advice

71. As the Company would remind investors throughout the Class Period, ACT DMD was designed as a "confirmatory" study, intended to ratify the Company's purported efficacy findings from the Phase 2b trial. Because of its refined enrollment criteria, the Phase 3 trial was trying to confirm the positive results from one of the subgroups whose data they analyzed post-hoc in the Phase 2b study.

72. Indeed, enrollment in ACT DMD, which was also known as "Study 020," was restricted to DMD patients who could still walk (ambulatory patients) aged 7-16 years old—those children that, based on the Phase 2b results, the Company predicted would demonstrate the strongest response to Translarna. The goal was to focus heavily on enrolling children in a specific subset of DMD patients in what the Company described as the "decline phase."

73. This design was selected based on the results of the Phase 2b study, from which the Company developed the belief that children who were able to walk less than 350 meters in 6 minutes were thought to be experiencing a less advanced form of the disease and were therefore more stable, but were still likely to show a significant decline over the course of 48 weeks. To

further power the study, PTC also eliminated boys in the advanced stages of the disease who were not able to walk.

74. By tailoring the ACT DMD study enrollment to these patients, PTC greatly increased the likelihood that it would provide sufficient evidence that Translarna is effective at treating nmDMD. However, in crafting these enrollment criteria, PTC also excluded many of its target customers to whom the Company would eventually seek approval to market and sell Translarna (*i.e.* the more stable children under the age of seven, as well as any patient whose muscular dystrophy was too advanced for them to achieve PTC's desired results).

75. Nonetheless, this strategy, by which the Company claimed it had "wrung out the risk," provided PTC with the best odds of demonstrating the drug's effectiveness in a small segment of the overall nmDMD patient population, even though the Company intended to seek approval of Translarna for the ***entire*** nmDMD population, including those who exhibited no demonstrated effect in clinical trials.

76. Armed with this information, in the spring of 2015, PTC submitted its draft statistical analysis plan and trial design to the FDA who then discussed the plan with the Company before providing final comments and signing off. What Defendants did not discuss with the FDA, and would later reveal, is that Defendants would use an unapproved post-hoc analysis as their "main analysis."

77. The ACT DMD trial design included the same benchmark goals as the Phase 2b. The primary goal of the study would be the same as the Phase 2b study, a clinically meaningful and statistically significant change in 6MWD at 48 weeks, compared to week 1. Like the Phase 2b trial, a clinically meaningful benefit was still defined at the threshold of 30 meters and the level of statistical significance was still the benchmark 0.05.

78. According to PTC's SEC Form 10-Q, filed on November 9, 2015, (after the ACT DMD results were released), the statistical analysis plan also included several pre-specified subgroups, including the key < 350 meter subgroup, which the Company designed the trial around. PTC described the < 350 meter subgroup "as a key subgroup based on the knowledge that ***350 meters represents a transition point for patients towards a more rapid decline in walking ability as supported by analysis from our Phase 2b study.***"

79. At least one other subgroup that was pre-specified for purposes of statistical analyses were patients who walked between 300 and 400 meters (the 300-400 meter subgroup) during the baseline walking test. PTC included this group "based on an increasing understanding of the sensitivity limitations of the six minute walk test as an endpoint in 48-week studies." Prior to the release of the ACT DMD results, the Company had not disclosed the existence of the 300-400 meter subgroup to the public.

80. The statistical analysis plan also included one "meta-analysis" that combined the "efficacy results from the ACT DMD ITT population and Phase 2b ambulatory decline phase subgroup." By including the meta-analysis, PTC greatly increased its chances of being able to present a statistically significant result to the FDA, due to the substantially larger patient population to draw data from. In other words, the meta-analysis would combine what the Company characterized as positive results from the Phase 2b trial and aggregated them with the overall results of the ACT DMD trial, in an effort to show Translarna's efficacy.

E. ACT DMD Failed to Confirm Any of the Results of the Phase 2b Trial

81. Defendants announced the results of the ACT DMD Phase 3 study in October 2015 (but did not disclose the full extent of the trial data). Once again, the study failed to show a statistically significant result for the primary endpoint. The primary endpoint analysis actually showed substantially ***worse*** results than the Phase 2b trial. Moreover, 6-minute walk distance for

the overall population was also worse than the Phase 2b result, and significantly below the Company's pre-specified threshold for evidencing a clinically meaningful benefit.

82. Importantly, the key < 350 meter subgroup around which the Company had designed the Phase 3 trial (and the primary basis for the Company's statements that the trial was "powered for success" and that they had "wrung out the risk") also failed to show a clinically meaningful benefit or statistical significance.

83. Although the Company acknowledged that the study did not meet the requisite level of statistical significance for the primary endpoint for the overall population, or for the key < 350 meter subgroup, PTC lauded the trial's positive results because they were able to demonstrate a clinically meaningful and statistically significant result for one small subgroup, the 300-400 meter subgroup.

84. Defendants also claimed that these positive trial results were supported by the "pre-specified meta-analysis of [their] combined Phase 2b and ACT DMD results." However, this result was not clinically meaningful (even if it may have been statistically significant), and thus did not meet the trial's primary clinical endpoint that the Company had chosen, with the FDA's assistance, to define success.

85. In an attempt to salvage the trial result, PTC also reported a second meta-analysis that cherry-picked what Defendants now knew was the highest performing subgroup in the Phase 3 trial, and combined it with patients who fit that definition in the completed Phase 2b trial. Unsurprisingly, this retrospective meta-analysis showed a very high clinically meaningful benefit and level of statistical significance.

86. But this second meta-analysis was not pre-specified by PTC prior to the start of the Phase 3 trial. The fact that this analysis was not pre-specified is key, because the fact that it

was developed only ***after*** the results of the trial were known and the clinical trial data was fully digested meant that it could have been, and was, designed for the specific purpose of finding a way to deem the failed trial a success and intentionally misleading the public (including the investing public) as to Translarna's actual efficacy. The emphases placed by the Company on these subgroups and meta-analyses, however, misleadingly gave investors the impression that PTC had been able to demonstrate in two pre-specified analyses (one subgroup, another meta-analysis) that Translarna provided a clinically meaningful result that was statistically significant to an extremely high degree.

87. Given that the Phase 3 trial population was designed around the subgroup that the Company claimed saw the greatest benefit from Translarna in Phase 2b, it should have produced stronger evidence of efficacy than the Phase 2b trial. Instead, while the Company's Phase 2b data purportedly showed that the treatment population saw ***some*** improvement over placebo that was not clinically meaningful (29.7 meters) and that even this sub-par improvement was not statistically significant, Phase 3 was only able to show that the overall ITT population that had been treated with Translarna saw roughly half the benefit purportedly observed in the prior trial, with even worse statistical significance.

88. While PTC focused investors' attention on the beneficial results in the 300 meter to 400 meter subgroup, the Company failed to address the fact that Phase 3 patients outside of this range (approximately 57% of the overall ITT population) ***saw no clinically meaningful or statistically significant benefit*** to treatment with Translarna. In other words, Translarna had not been shown to work in patients outside of the small 300-400 meter subgroup (and indeed showed even less efficacy among the overall population than data from Phase 2b trial).

89. Moreover, it is clear that the ACT DMD trial did ***not*** confirm Translarna's efficacy. To the contrary, the Phase 3 trial yielded results that actually undermined the aspects of the Company's Phase 2b study, which PTC had previously leveraged as the very reason why the Phase 3 study was "powered for success." In addition to significantly worse results in the overall patient population, the Phase 3 trial was also unable to "confirm" a clinically meaningful benefit or statistical significance in the < 350 meter subgroup, the very group the entire study was predicated on.

90. Nonetheless, during the conference call announcing the ACT DMD results, Defendants consistently reassured investors that the results demonstrated the type of efficacy that would be required to secure FDA review and approval. During the call, Defendant Peltz told investors ***"[t]he results from ACT DMD trial showed consistent evidence of the clinical benefit of Translarna for individuals with nonsense mutation Duchenne muscular dystrophy, and it's impact on the course of the disorder, and the quality of life for those boys and young men."*** This statement, and others like it, created the inescapable impression among investors that even with substandard results from both trials, the evidence would be sufficient to file a NDA with the FDA that would subsequently be approved.

F. Submission of ACT DMD Trial Data, Which Did Not Demonstrate Translarna's Efficacy as Defendants Had Predicted, Completed PTC's Rolling Submission for Translarna's NDA

91. In January 2016, three months after the release of the initial Phase 3 trial results, the Company announced that it had completed its NDA submission. This second NDA, which was for full—not conditional—approval, was supported with data from both the Phase 3 and Phase 2b clinical trials, and sought a broad-label approval, meaning that it was seeking approval for treatment of all nmDMD patients, regardless of how far the disease had progressed.

92. In support of its NDA, PTC relied on the purportedly favorable findings from the 300-400 meter subgroup and the pre-specified meta-analysis which combined the data from the Phase 2b and Phase 3 trials, and which the Company claimed "provide[d] substantial evidence of the effectiveness of Translarna . . . for the treatment of nmDMD." PTC's inclusion of the meta-analyses would purportedly satisfy the FDA requirement that drug sponsors demonstrate "substantial evidence" that a drug is safe and effective, "based on the results of at least two pivotal trials."

93. PTC also relied on a post-hoc statistical analysis of the 300-400 meter subgroup from the ACT DMD trial to claim it had demonstrated that Translarna was effective at treating nmDMD. This analysis, which was not pre-specified, allowed the Company to easily discard data from almost 60% of the study population, but was the Company's best evidence to support the impression that Translarna actually worked.

94. PTC's inclusion of the two meta-analyses would purportedly satisfy the FDA requirement that drug sponsors demonstrate "substantial evidence" that a drug is safe and effective, "based on the results of at least two pivotal trials," even though neither of the two trials was able to meet their primary endpoints. The reasonable investor, however, unfamiliar with the regulatory threshold for demonstrating drug efficacy (and the statistical analyses requires to show such proof), would have been unable to determine the true import of the trial data, or understand that the Company was underrepresenting the known elevated risks of FDA rejection.

95. Moreover, notwithstanding the fact that the Company was able to manufacture some impressive results through the application of post-hoc analyses that the FDA would deem improper, Defendants fully understood that, in order for the FDA to accept the Company's NDA submission, a demonstration of efficacy in only a small sub-segment of the population would be

insufficient for full approval. For example, during an explanation of why the ACT DMD study was "well-powered for success," at an August 2015 Wedbush PacGrow Healthcare Conference, Defendant Kovacs readily acknowledged that "clearly the drug ***should*** have benefit across the whole population."

G. The FDA Refused to Accept PTC's NDA for Filing

96. On February 22, 2016, PTC received a "Refuse-to-File" letter (the "2016 RTF") from the FDA regarding the Company's NDA for Translarna. While not publically available, according to PTC, the letter advised PTC that the NDA was "***not sufficiently complete to permit a substantive review***" of the application. Defendants went on to state "there were really two bases . . . that were outlined in the letter; ***the first of which was that both the Phase 2b and Phase 3 studies had failed and therefore did not demonstrate substantial evidence of effectiveness*** and then secondly that the application did not sufficiently describe the abuse potential of the drug."

97. Analysts reacted to the announcement accordingly. A February 23, 2016 analyst report published during the trading day by Anupam Rama from J.P. Morgan stated that PTC's shares at that point were already "down 40%+ and justifiably so" given analyst's concerns surrounding the FDA's RTF letter for Translarna. The report went on to question the Company's credibility as a result of the unexpected RTF letter. "Currently, there are more questions than answers, including 1) nature [and] details of RTF, 2) the possibility of additional pivotal trial work in DMD for US approval . . . and 5) credibility of management."

98. Likewise, analysts from RBC Capital Markets described the "sell-off as justified" in an analyst report published on February 24, 2016. The report stated, "[w]hile there were no details disclosed about the contents of the Refuse to File letter, we view the market sell-off as

justified given that 1) [Translarna] failed its confirmatory Phase III trial . . . and 2) FDA has recently shown it will not approve drugs it does not believe work."

99. Following the initial announcement, PTC continued to reveal details about the RTF to investors. In a press release dated February 29, 2016, PTC acknowledged that the RTF indicated the FDA's view that "both the Phase 2b and ACT DMD trials were negative and do not provide substantial evidence of effectiveness," and that "certain of the [C]ompany's adjustments to the ACT DMD study [were] post-hoc and therefore not supportive of effectiveness." According to the press release, the RTF letter also stated that PTC's NDA "did not contain adequate information regarding the abuse potential of Translarna, a requirement for new molecules that cross the blood-brain barrier."

100. Then, on February 29, 2016, PTC hosted a conference call with analysts to discuss the results of Q4 2015 and provide details on the FDA's RTF. Defendants Peltz participated on the call and explained some of the details regarding the FDA's rejection of the NDA. In his prepared remarks, Defendant Peltz explained the FDA's viewpoint, noting, "***[f]irst, in the view of the FDA, both the Phase IIB and Phase III ACT DMD trials were negative and did not provide substantial evidence of effectiveness. The FDA also characterized certain of our adjustments to the ACT DMD study as post-hoc and therefore not supportive of effectiveness.***"

101. After delivering his prepared remarks, the first question asked related to the FDA's characterization of some analyses as post-hoc when, according to the analyst, past Company comments indicated "that the stat plan was submitted to the FDA earlier in 2015." In response, Defendant Peltz claimed that, although they had submitted a statistical analysis plan, which included subgroups, the FDA never commented on subgroups.

102. During the very same response, however, Peltz revealed that, based on the RTF letter, the FDA believed PTC's NDA was insufficient because "***relying on the [300-400 meter] subgroup as the main analysis is considered as a post hoc adjustment***" and that the Company's submission therefore "***eliminates data from a majority of enrolled patients.***"

103. In full, Peltz responded:

> On the -- overall on the subgroup analysis, the FDA characterized -- first of all the FDA characterized our NDA as not supportive of effectiveness. This would include the subgroup analysis.
>
> So I think in terms of the history it's important to remember that we included the subgroup in our statistical analysis plan which we submitted in the spring of 2015. At that time the FDA commented on our statistical analysis plan but had no comments on our subgroups.
>
> We submitted the final statistical analysis plan to the FDA before unblinding the ACT DMD study. However in the RTF letter the FDA characterized that ***PTC proposed a post hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients.*** So we need further discussion with the FDA to understand their current perspective on our subgroup analysis.
>
> We believe the FDA's perspective in the RTF letter may be that ***although we've pre-specified the subgroup, relying on the subgroup as the main analysis is considered as a post hoc adjustment*** and we'll be talking to them further on this point.

104. Following the call, several analysts downgraded the stock citing the Company's lack of transparency and eroded credibility. For example, one analyst report entitled "Downgrading As Translarna Becomes a Show-Me Story" stated "[w]e believe there is little transparency on FDA communications and EU pricing proceedings to give investors comfort, ***and importantly, no additional transparency forthcoming despite eroded credibility***. We are downgrading to Market Perform as PTCT has become a show-me story."

105. The analyst report also highlighted the recklessness of PTC's application given the substantial leeway the FDA grants to orphan-drug developers.

> [T]he issue was the fact that FDA deemed the baseline 6MWT subpopulation analysis as "post-hoc" or otherwise deficient in statistical-meaning. PTCT does assert that it submitted the subpopulation analysis in its statistical analysis plan, which FDA did not comment on. ***We note, again, that PTCT did not meet with FDA after ACT-DMD results were released and before completion of the rolling NDA. The level of miscommunication (or lack of communication) with the agency suggests a major breakdown of the regulatory communication process. It is also at odds with FDA's very public message to orphan drug developers to talk to them "early and often".***
>
> ***Our concern around the lack of quality communication is only deepened by the second point of the RTF: the inadequacy of the abuse potential. We believe this an issue addressed by a large proportion of sponsors, and is therefore a relatively straightforward, almost mundane, regulatory requirement that somehow slipped through the cracks of the Translarna filing package.***

106. Another analyst report, from Anupam Rama of J.P. Morgan, lowered the price target for the Company and explained:

> More importantly, on the call, PTC provided some color around the RTF letter last week, however several questions remain related to the path forward in the US and additional clinical trial work cannot be ruled out at this point. Despite our earlier move to Neutral [ ], ***we were somewhat surprised by FDA's response regarding the pre-specified subgroup analyses in the ACT DMD study, which will not help growing management credibility concerns.***

107. On August 4, 2016, the Company announced in a press release that it had appealed the FDA's "Refuse to File (RTF) letter issued on February 22, 2016 with respect to the company's New Drug Application (NDA) for Translarna for the treatment of nmDMD." On November 2, 2016, the Company announced that the appeal was denied. The Company stated that it "intends to continue the appeal to higher levels of the FDA."

DEFENDANTS' FALSE AND MISLEADING STATEMENTS

A. November 6, 2014

108. The Class Period begins on November 6, 2014, when PTC filed its Form 10-Q for the 2014 third quarter (the "3Q 2014 Form 10-Q") with the SEC and announced that the Company would begin submitting the information for the 2016 NDA on a rolling basis. The 3Q 2014 Form 10-Q, which was signed by Defendant Kovacs, touted the benefits of a Rolling Review of its NDA for Translarna for nmDMD, without any mention of the substantial risk that ACT DMD's failure to meet its primary clinical endpoint would result in the FDA's outright rejection of the NDA as facially inadequate:

> We also plan to initiate a rolling . . . NDA, with the . . . FDA, for Translarna as a treatment for nmDMD. We currently anticipate that we will commence the submission process before the end of 2014. We believe this process gives the FDA an opportunity to conduct a meaningful review of most of the segments of our NDA, ahead of reviewing our Phase 3 ACT DMD data. We expect that the submission of the ACT DMD data will complete our rolling NDA.

109. That same day, after the market closed, PTC hosted a conference call with analysts to discuss the Company's financial results and the status of the ACT DMD clinical trial. Defendant Peltz participated on the call and continued to build expectations concerning the FDA's review of the Translarna NDA. In his prepared remarks, Peltz explained to the market that, not only would the FDA be reviewing the Translarna NDA, but that the FDA told PTC that it would be reviewed on an expedited basis:

> Regarding regulatory approval of Translarna for DMD patients in the US, we've been in dialog with the FDA. We now intend to initiate a rolling NDA submission by the end of this year for the approval of Translarna for DMD in the US. This process gives the FDA an opportunity to conduct meaningful review of most of the segments of our NDA ahead of reviewing our Phase 3 ACT DMD data. ***We expect that the submission of this confirmatory Phase 3 data will complete our rolling NDA.*** Based on our discussions

> with the FDA, we expect that our rolling NDA will be rapidly reviewed for potential approval within the first half of 2016.[12]

110. Similarly, in response to a question from an analyst concerning the US commercial launch timeframe, Peltz stated:

> I think that the way we're thinking about this is that, as I said, the discussions with the FDA that will get the various segments of the NDA in and then on completion of ACT DMD trial, the data will come out. ***We'll then expeditiously get it in and it's our hope and I think in our dialog with them, given the severe unmet medical needs that this would be rapidly reviewed on that, that this would expect in terms of the approval to move it up potentially after six months. So I think we're thinking about is that we would think that this can be a launch within the first half of 2016.***

111. The statements set forth above in Paragraphs 108-110 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. It was likewise materially misleading for Defendants to describe the ACT DMD trial as "confirmatory" while omitting the fact that the FDA would require PTC to demonstrate Translarna's efficacy more sufficiently than the Company had in its Phase 2b trials; the Company's meetings and discussions with the FDA following the 2011 RTF gave Defendants unique insight into what the FDA would require in the Company's Translarna Phase 3 NDA.

112. Moreover, Defendant Peltz's statement that the Company hoped its "dialogue" with the FDA would lead to the NDA being "rapidly reviewed," and "launch within the first half

[12] All emphases set forth in Paragraphs 108-151 are added, and indicate the specific portion of each statement alleged to have been false and/or misleading, whether by reason of affirmative misstatement or omission.

of 2016" was also false and misleading because it omitted to state the significant likelihood that the Company's NDA would not be reviewed ***at all***, if the ACT DMD results did not "confirm" the efficacy of Translarna as purportedly demonstrated by the results of the Phase 2b trial.

B. January 15, 2015

113. On January 15, 2015, PTC representatives attended a conference called the JPMorgan Healthcare Conference. In his opening remarks, Defendant Peltz stated: "I expect [] a number of important milestones [in 2015]. ***We have the confirmatory trial for Duchenne muscular dystrophy ongoing. That will allow us then to sell it in the United States, where we expect each trial to be completed this year and next year that we get approval in the US.***"

114. In looking back to discuss the results from the Translarna Phase 2b Trial and their import for the Company, Peltz also noted:

> ***And a confirmatory trial to be able to get it in the US is well underway, and it's already fully enrolled. What we did is we used the learnings from the previous study to really wring out the risk in the current study.***

115. Analysts following the Company responded positively to the Company's assurances that risk in the ACT DMD trial had been "wrung out" by taking into account the purported lessons the Company learned from the Phase 2b study. For example, an analyst report from RBC Capital Markets stated under the heading "Key Questions":

> 1. Will the Phase III ACT DMD trial succeed, and how will the changes to the patient enrollment criteria affect its probability of success?
>
> Given the new trial design changes, we believe that the Phase III trial would work and meet its endpoint. Having received conditional approval in the EU for the treatment of nmDMD, PTC Therapeutics needs positive results in its Phase III ACT DMD trial for both full EU approval and for approval in the US. The new Phase III trial design builds on the strengths of the Phase II data, since it will only enroll patients in the ambulatory decline phase, which was the group that benefited most in the Phase II trial.

> Patients who may not experience rapid decline, such as younger patients and patients with a baseline ambulatory ability above a certain threshold will now be excluded from the trial.

116. The statements set forth above in Paragraphs 113-114 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. It was likewise materially misleading for Defendants to describe the ACT DMD trial as "confirmatory" while omitting the fact that the FDA would require PTC to demonstrate Translarna's efficacy more sufficiently than the Company had in its Phase 2b trials; the Company's meetings and discussions with the FDA following the 2011 RTF gave Defendants unique insight into what the FDA would require in the Company's Translarna Phase 3 NDA.

117. Specifically, Defendants' statement that "we used the learnings from the previous study to really wring out the risk in the current study" was false and/or materially misleading when made, or was rendered misleading by omitting material information necessary to make the statement not false and/or misleading because Defendants intentionally minimized the substantial risk that the ACT DMD trial would fail to meet its primary clinical endpoints, and thus that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. Defendants knew at the time, but failed to tell the investing public, that the design of the ACT DMD trial had just as much of a risk of failure as had the Phase 2b trial, and that the design of ACT DMD had not minimized any risk of negative outcomes.

C. March 9, 2015

118. On March 9, 2015, PTC representatives attended a conference called the ROTH Capital Annual Conference. Tuyen Ong, PTC's Senior Vice President, Head of Clinical Development and Translational Research, spoke at the ROTH Conference, discussing the positive results of the Phase 2b trial and reiterating to the market why the "confirmatory" ACT DMD trial was different and would allow for FDA review and approval:

> ***I think we talked about the confirmatory studies really being enriched and sort of enhanced and somewhat we've wrung out the risk of the confirmatory study based on the learnings of the second study.*** I think ultimately it's really dependent on the data; is there a clinical benefit, is it statistically significant. So, a lot of moving factors around this. But I think based upon what we've done to address some of the sort of risk that we talked about, we feel pretty confident that we've addressed all the sort of items that we can take care of at this stage.

119. The statement set forth above in Paragraph 118 was false and/or materially misleading when made, or was rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. It was likewise materially misleading for Defendants to describe the ACT DMD trial as "confirmatory" while omitting the fact that the FDA would require PTC to demonstrate Translarna's efficacy more sufficiently than the Company had in its Phase 2b trials; the Company's meetings and discussions with the FDA following the 2011 RTF gave Defendants unique insight into what the FDA would require in the Company's Translarna Phase 3 NDA.

120. Specifically, Defendants' statement that "being enriched and sort of enhanced and somewhat we've wrung out the risk of the confirmatory study based on the learnings of the

second study" was false and/or materially misleading when made, or was rendered misleading by omitting material information necessary to make the statement not false and/or misleading because Defendants intentionally minimized the substantial risk that the ACT DMD trial would fail to meet its primary clinical endpoints, and thus that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. Defendants knew at the time, but failed to tell the investing public, that the design of the ACT DMD trial had just as much of a risk of failure as had the Phase 2b trial, and that the design of ACT DMD had not minimized any risk of negative outcomes.

D. May 6, 2015

121. On May 6, 2015, PTC representatives attended a conference called the Deutsche Bank Health Care Conference. At this conference, Defendant Kovacs reassured the market that the Company was monitoring the trial and discussed the likelihood that the FDA would approve Translarna if the ACT DMD Trial outcome was the same as the Translarna Phase 2b Trial:

> The data would actually [] have to be substantially worse than the last Phase II study to miss the 0.05 significance, if that makes any sense. . . . ***[O]bviously we've tried to mitigate as much risk as possible in this study by a lot of the care around reducing the enrollment criteria and try to control for the patients that were at least enrolled in the study,*** but in addition to that, we're trying to think about the statistical analysis plan that gets submitted in terms of what are pre-defined subgroups that we can define. In case the overall population has a near miss, can we hit it in a predefined subgroup? ***So we're doing that (inaudible) de-risk the outcome***.

122. The statements set forth above in Paragraph 121 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially

insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review.

123. Specifically, Defendants' statements that "obviously we've tried to mitigate as much risk as possible in this study by a lot of the care around reducing the enrollment criteria and try to control for the patients that were at least enrolled in the study" and that they were "doing that (inaudible) de-risk[ed] the outcome" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants intentionally minimized the substantial risk that the ACT DMD trial would fail to meet its primary clinical endpoints, and thus that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. Defendants knew at the time, but failed to tell the investing public, that the design of the ACT DMD trial had just as much of a risk of failure as had the Phase 2b trial, and that the design of ACT DMD had not minimized any risk of negative outcomes.

E. May 12, 2015

124. On May 12, 2015, representatives from PTC, including Defendant Kovacs, attended the Bank of America Merrill Lynch Health Care Conference. During the conference, Defendant Kovacs continued to tout the Company's confidence in the ACT DMD trial:

> ***[I]f you think about what have we done and refined for this study versus the prior Phase 2 study that gives us such a high degree of confidence in the likelihood of a positive outcome in this study later this year***, one, we really refine the enrollment criteria in this study versus the last study. We take boys only seven years up to 16, whereas before, we took five and six year old boys.

125. The statement set forth above in Paragraphs 124 was false and/or materially misleading when made, or were rendered misleading by omitting material information necessary

to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. Defendants knew at the time, but failed to tell the investing public, that the design of the ACT DMD trial had just as much of a risk of failure as had the Phase 2b trial, and that there was no basis to believe that the trial's outcomes would positively demonstrate efficacy pursuant to its primary clinical endpoints.

F. October 15, 2015

126. On October 15, 2015, PTC hosted a conference call to discuss the results of the ACT DMD trial. In his opening remarks, Defendant Peltz touted the success of the "two large placebo-controlled trials" and the efficacy they purportedly demonstrated:

> Turning to the results of ACT DMD trial on slide 5, we are very pleased that ***the totality of the Translarna results demonstrate clinical benefit for DMD. These include ITT results, the pre-specified subgroup results, and pre-specified meta-analysis***, as well as secondary timed-function tests and the North Star Assessment.

127. Peltz also touted the success of the trial because of the "meta-analysis," which he claimed "demonstrates a clinically relevant benefit in preserving muscle function, and in changing the course of disease for Translarna treated DMD patients across all primary and secondary endpoints over a 48-week period."

128. Defendant Peltz also noted during the call that:

> ***The totality of the data for Translarna demonstrates clinical benefit across primary and secondary endpoints. We have pre-specified the key subgroup for analysis and the meta-analysis, both of which show Translarna had a clinically meaningful benefit for DMD patients. The results from ACT DMD trial showed consistent evidence of the clinical benefit of Translarna for individuals with nonsense mutation Duchenne muscular***

> ***dystrophy, and it's impact on the course of the disorder, and the quality of life for those boys and young men.***

129. In response to questions from several analysts, Defendant Peltz explicitly emphasized that Defendants had discussed the use of subgroups with the FDA, and strongly implied that the Company's statistical analyses that would be submitted with its NDA for Translarna had previously been approved by the FDA:

> Joel Beatty - Citigroup – Analyst
>
> Hi, thanks for taking the question. So have you had discussions with the FDA on the degree of consideration they might give to pre-specified meta-analysis? And if so, can you provide any more information on that?
>
> Stuart Peltz - PTC Therapeutics Inc. – CEO
>
> Sure, yes. Thanks for that question. ***The pre-specified meta-analysis was in our statistical analysis plan, which we had discussions with with[sic] the FDA. This was in part, part of the pre-specified plan. So they are well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure. I should make one other point, is that we have had conversations with the FDA. One thing they even said we could say publicly, this is very important for them, and that they are trying to rapidly, giving this a high priority, give the highest priority in order to try and get drugs to these patients.***

130. In closing, Defendant Peltz reiterated that the ACT DMD results purportedly "confirmed" that Translarna effectively treated nmDMD:

> Well, thanks all for the questions, and thanks for joining us today. ***We are proud to have confirmed the benefit of Translarna for the DMD patients.*** After over 17 years of effort, this is a rewarding moment for everyone. Again, we are very proud, and we wish you all to have a good evening. Thanks much.

131. Following the call, analysts credited the Company's statements that the ACT DMD confirmed the clinical benefit of Translarna and was poised for approval by the FDA, based on the Company's strong results. One analyst report noted "the ***critical point*** is that

Translarna is an approvable agent from a regulatory perspective, given 1) totality of efficacy data, including supportive secondary endpoints, 2) no major safety concerns, and 3) significant unmet need in the disease."

132. The statements set forth above in Paragraphs 126-130 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. It was likewise materially misleading for Defendants to describe the ACT DMD trial as "confirmatory" while omitting the fact that the FDA would require PTC to demonstrate Translarna's efficacy more sufficiently than the Company had in its Phase 2b trials; the Company's meetings and discussions with the FDA following the 2011 RTF gave Defendants unique insight into what the FDA would require in the Company's Translarna Phase 3 NDA.

133. Defendants' statements that the "totality of the Translarna results demonstrate clinical benefit for DMD" and "[w]e are proud to have confirmed the benefit of Translarna for the DMD patients" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants were aware and/or deliberately reckless in failing to know that, "the totality of the results" did not "confirm[] the benefit of Translarna for DMD patients," but were actually less supportive of a finding of efficacy than the Phase 2b results, and that such outcome was facially inadequate to support a substantive review by the FDA.

134. Furthermore, Defendants' statements that "the totality of the Translarna results demonstrate clinical benefit for DMD" and that the clinical trial "confirmed the benefit of Translarna for the DMD patients" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because the meta-analysis results cited to support their claimed efficacy only applied to a small subgroup of nmDMD patients, and was not pre-specified in the Company's statistical analysis plan. As a result, Defendants knew, or were reckless in not knowing that the meta-analysis would be facially insufficient to form a complete application that would be filed by the FDA. In fact, Defendants knew or were reckless in not knowing that relying on the 300-400 meter subgroup as the main analysis would be considered a post-hoc adjustment by the FDA and that the Company's submission eliminated data from a majority of enrolled patients, thereby making the NDA submission inadequate on its face.

135. Moreover, Defendants' statements about the viability of the meta-analysis results as "demonstrat[ing] a clinically relevant benefit" and "changing the course of the disease for Translarna treated DMD patients across all primary and secondary endpoints" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because the meta-analysis results cited to support their claimed efficacy only applied to a small subgroup of nmDMD patients, and was not pre-specified in the Company's statistical analysis plan. As a result, Defendants knew, or were reckless in not knowing that the meta-analysis would be facially insufficient to form a complete application that would be filed by the FDA.

G. November 9, 2015

136. On November 9, 2015, the Company filed a Form 8-K with the SEC including a press release entitled "PTC Therapeutics Reports Third Quarter 2015 Financial Results and

Provides Corporate Update and Reviews Key Findings from ACT DMD." The Company therein continued to tout the Company's expectations of FDA review and approval of Translarna for nmDMD:

> ***ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy.*** On October 15th, PTC announced results from the Phase 3 ACT DMD clinical trial of Translarna in patients with nmDMD. The totality of the clinical data from two large, placebo-controlled clinical trials across over 400 patients ***demonstrates Translarna's ability to slow disease progression***. Today, on PTC's quarterly investor call the Company will review key findings from the ACT DMD clinical trial.

137. On November 9, 2015, PTC also filed its Form 10-Q for the third quarter of 2015 (the "3Q 2015 Form 10-Q") with the SEC. The 3Q 2015 Form 10-Q, which was signed by Defendant Kovacs, stated, in relevant part:

> ***[W]e believe that the results of ACT DMD and the totality of clinical data across our two large, randomized, placebo-controlled trials (ACT DMD and our prior Phase 2b study, Study 007), provide substantial evidence of the effectiveness of Translarna and demonstrate a clinically meaningful benefit of Translarna for the treatment of nmDMD.***

138. That same day, after the market closed, PTC hosted a conference call with analysts to discuss the third quarter 2015 results, during which, Defendant Peltz implicitly told the market that Translarna had demonstrated efficacy sufficiently for FDA approval:

> [T]he goal is to show efficacy with given endpoints in the limited window of a 48-week clinical study. ***We see this in ACT DMD.***
>
> * * *
>
> . . . ***the totality of clinical data confirmed Translarna's ability to slow disease progression for patients with DMD.***

139. That same day analysts published reports crediting the Company's laudatory statements from the call, and noting their expectation that the FDA would approve the drug

based on the reported results. One analyst from Oppenheimer noted that "PTC discussed ACT-DMD Phase 3 data in nmDMD patients, which demonstrated positive trends, and strong concordance across multiple endpoints. We continue to expect approval of Translarna, and broad utilization considering the favorable risk/benefit profile demonstrated by ACT-DMD."

140. The statements set forth above in Paragraphs 136-138 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants failed to sufficiently disclose the substantial risk that the Translarna NDA submission would be rejected as facially insufficient by the FDA, thereby preventing the filing of the Company's 2016 NDA for substantive review. It was likewise materially misleading for Defendants to describe the ACT DMD trial as "confirmatory" while omitting the fact that the FDA would require PTC to demonstrate Translarna's efficacy more sufficiently than the Company had in its Phase 2b trials; the Company's meetings and discussions with the FDA following the 2011 RTF gave Defendants unique insight into what the FDA would require in the Company's Translarna Phase 3 NDA.

141. Furthermore, Defendants' statements that "totality of the clinical data" did not "demonstrate clinical benefit for DMD" or "confirm Translarna's ability to slow disease progression for patients with DMD," and that the "ACT DMD results confirm clinical benefit of Translarna in [nmDMD]" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because the meta-analysis results cited to support their claimed efficacy only applied to a small subgroup of nmDMD patients, and was not pre-specified in the Company's statistical analysis plan. As a result, Defendants knew, or were reckless in not knowing that the meta-

analysis would be facially insufficient to form a complete application that would be filed by the FDA. In fact, Defendants knew or were reckless in not knowing that relying on the 300-400 meter subgroup as the main analysis would be considered a post-hoc adjustment by the FDA and that the Company's submission eliminated data from a majority of enrolled patients, thereby making the NDA submission inadequate on its face.

142. Moreover, Defendants' statements regarding the totality of the data supporting Translarna's efficacy were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants underrepresented the risk that the Company's NDA submission, which only showed statistical significance for one small subgroup, and in a meta-analyses (that was not pre-specified) of that subgroup across both Phase 2b and Phase 3 trials would be facially insufficient to support an approval for broad label use of Translarna for all nmDMD patients. In fact, Defendants knew, or were deliberately reckless in not knowing, that only showing statistical significance for this small subgroup and in meta-analyses would be facially inadequate to support a complete application that would be reviewed by the FDA.

H. November 18, 2015

143. On November 18, 2015, representatives from PTC, including Defendant Kovacs, attended the Stifel Healthcare Conference. Kovacs spoke on PTC's behalf, again touting the expectation that PTC's Translarna would be reviewed quickly and approved by the FDA:

> And the big picture about our data is and what will be part of our argument to both the regulatory authorities in the US and Europe is that ***the consistency of the results now seen across two of the largest placebo-controlled Phase 3 studies ever done in the disease, the totality of the data support the clinical benefit and*** certainly the risk-benefit profile of the drug ***in favor of an approval*** and getting something to these kids.

144. The statement set forth above in Paragraph 143 was false and/or materially misleading when made, or was rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants were aware and/or deliberately reckless in failing to know that, according to the FDA "the consistency of the results" and the "totality of the data" did not "support the clinical benefit . . . in favor of an approval" but in fact, were facially inadequate to support a substantive review by the FDA. Specifically, Defendants were aware of, but failed to inform investors about, additional efficacy requirements set forth in the Company's discussions with the FDA following the first RTF of the Company's 2011 NDA and in specific guidance from the FDA related to the development of drugs for treatment of DMD that contradicted the Company's statements implying that the data was sufficient to support FDA approval, much less substantive review.

145. Furthermore, Defendants' statements that "the consistency of the results now seen across two of the largest placebo-controlled Phase 3 studies ever done in the disease, the totality of the data support the clinical benefit and certainly the risk-benefit profile of the drug in favor of an approval" were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because the meta-analysis results cited to support their claimed efficacy only applied to a small subgroup of nmDMD patients, and was not pre-specified in the Company's statistical analysis plan. As a result, Defendants knew, or were reckless in not knowing that the meta-analysis would be facially insufficient to form a complete application that would be filed by the FDA. In fact, Defendants knew or were reckless in not knowing that relying on the 300-400 meter subgroup as the main analysis would be considered a post-hoc adjustment by the FDA and that

the Company's submission eliminated data from a majority of enrolled patients, thereby making the NDA submission inadequate on its face.

146. Moreover, Defendants' statements regarding the consistency of the data supporting Translarna's efficacy were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants underrepresented the risk that the Company's NDA submission, which only showed statistical significance for one small subgroup, and in a meta-analyses (that was not pre-specified) of that subgroup across both Phase 2b and Phase 3 trials would be facially insufficient to support an approval for broad label use of Translarna for all nmDMD patients.

I. December 9, 2015

147. On December 9, 2015, representatives from PTC, including Defendant Kovacs, attended the Oppenheimer Healthcare Conference. At the conference, Kovacs stated:

> ***[O]ur intention today is for filing for full approval on the basis of two large well-controlled studies that all point to safety and efficacy for a risk-benefit profile in favor of the drug.***

148. The statement set forth above in Paragraph 147 was false and/or materially misleading when made, or was rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants stated that the ACT DMD study results were sufficient to submit a full and complete NDA to the FDA for approval, when in fact, the Company knew or was deliberately reckless in failing to know that the trial data from the ACT DMD study would be facially insufficient to form a complete application that would be reviewed by the FDA.

149. Furthermore, Defendants' statement that "two large well-controlled studies that all point to safety and efficacy" was false and/or materially misleading when made, or were

rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants underrepresented the risk that the Company's NDA submission, which only showed statistical significance for one small subgroup, and in a meta-analyses (that was not pre-specified) of that subgroup across both Phase 2b and Phase 3 trials would be facially insufficient to support an approval for broad label use of Translarna for all nmDMD patients. In fact, Defendants knew, or were deliberately reckless in not knowing, that only showing statistical significance for this small subgroup and in meta-analyses would be facially inadequate to support a complete application that would be reviewed by the FDA. In fact, Defendants knew or were reckless in not knowing that relying on the 300-400 meter subgroup as the main analysis would be considered a post-hoc adjustment by the FDA and that the Company's submission eliminated data from a majority of enrolled patients, thereby making the NDA submission inadequate on its face.

J. January 13, 2016

150. On January 13, 2016, representatives from PTC attended the JPMorgan Healthcare Conference. PTC was represented by Defendant Peltz who continued to tout the likelihood of PTC's FDA approval of Translarna for nmDMD based on the results on the ACT DMD trial:

> ***So you can see in two large studies where we used the six-minute walk test as the primary endpoint, we saw a benefit both in the primary endpoint as well as secondary endpoints. And in prespecified subgroups, we saw more robust effects being observed, both the primary and secondary endpoints***. So consistent data in two independent studies.
>
> One of the things we've noticed they asked for was a sensitivity analysis, and that while you have prespecified subgroups, ***if you go beyond those, does the data still show clinically meaningful differences? And it does both in the primary and secondary endpoints.***

151. During the conference, Defendant Peltz also discussed the meta-analysis, heavily emphasizing the consistency of both trials in demonstrating Translarna's efficacy:

> ***In the meta-analysis, where you combine the results, you see both in the six-minute walk distance as well as the time function tests, you see clinically meaningful and statistically significant improvements with Translarna over placebo.*** So, when you look at -- and I think here's a nice example, I'm looking at the forest plot, ***looking at the totality of the results, you see quite clearly that treating patients with Translarna always was better -- always showed efficacy versus placebo, no matter whether you look at the six-minute walk test or the time function test.***
>
> ***And really it's consistent with the totality of the data, demonstrating that this drug was efficacious. So, I think we've checked that box.***

152. The statements set forth above in Paragraphs 150-151 were false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants stated that the ACT DMD study results were sufficient to submit a full and complete NDA to the FDA for approval, when in fact, the Company knew or was deliberately reckless in failing to know that the trial data from the ACT DMD study would be facially insufficient to form a complete application that would be reviewed by the FDA.

153. Furthermore, Defendants' statements that the data showed clinically meaningful effects even outside of the trial's subgroups was false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because the ACT DMD trial did ***not*** meet its primary clinical endpoints or other ITT analyses to demonstrate a clinically meaningful benefit of treatment to a statistically significant level.

154. Moreover, Defendants' statements regarding the "basis of two large well-controlled studies that all point to safety and efficacy" were false and/or materially misleading

when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading because Defendants underrepresented the risk that the Company's NDA submission, which only showed statistical significance for one small subgroup, and in a meta-analyses (that was not pre-specified) of that subgroup across both Phase 2b and Phase 3 trials would be facially insufficient to support an approval for broad label use of Translarna for all nmDMD patients.

155. These statements were also false and/or materially misleading when made, or were rendered misleading by omitting material information necessary to make the statements not false and/or misleading, because Defendants underrepresented the risk that the Company's NDA submission, which only showed statistical significance for one small subgroup, and in a meta-analyses (that was not pre-specified) of that subgroup across both Phase 2b and Phase 3 trials would be facially insufficient to support an approval for broad label use of Translarna for all nmDMD patients.

THE TRUTH EMERGES

156. The truth about the Company's failed confirmatory trial and unsuccessful second NDA for Translarna was revealed on February 23, 2016 when the Company disclosed that the FDA issued a RTF to PTC for the Translarna 2016 NDA. As a result, the FDA would not be reviewing any of the data in the actual NDA and rejecting it outright for being inadequate on its face.

157. PTC explained "the letter indicated from a high level that the application was not substantially complete to permit a review." Defendant Peltz went on to state that "there were really two bases . . . that were outlined in the letter; the first of which was that both the Phase 2b and Phase 3 studies had failed and therefore did not demonstrate substantial evidence of

effectiveness and then secondly that the application did not sufficiently describe the abuse potential of the drug."

158. As a result of this news, the price of PTC stock fell from its $28.26 closing price on Monday, February 22, 2016, to $10.84 at the close of trading on Tuesday, February 23, 2016, a loss of $17.42 per share, or 61.6%.

159. Analysts were shocked by the announcement, and characterized the huge stock drop as justified given the Company's credibility concerns. For example one analyst from RBC Capital Markets described the "sell-off as justified" in an analyst report published on February 24, 2016. The report stated, "[w]hile there were no details disclosed about the contents of the Refuse to File letter, we view the market sell-off as justified given that 1) [Translarna] failed its confirmatory Phase III trial . . . and 2) FDA has recently shown it will not approve drugs it does not believe work." In a section entitled "Downgrading to Sector Perform: FDA will not approve a drug it doesn't believe works," the RBC analyst report detailed why RBC was downgrading the stock based on the implications of the RTF letter:

> We anticipate more clarity on or before their earnings call scheduled for Monday, February 29 after market close. However, ***given 1) two failed trials in the DMD program, one of which was supposed to be the confirmatory one, and 2) FDA's recent tone, as seen in the drisapersen and eteplirsen cases, we believe that [Translarna] will have a very difficult time getting approved in the US. We have therefore removed all US DMD revenue from our model and we downgrade PTCT to Sector Perform and lower our price target to $11/share.***

160. Following the initial announcement, PTC continued to reveal details about the FDA's RTF letter to investors. In a press release dated February 29, 2016, PTC acknowledged that the RTF letter the FDA indicated that, in its view, "***both the Phase 2b and ACT DMD trials were negative and do not provide substantial evidence of effectiveness***" and that "***certain of the [C]ompany's adjustments to the ACT DMD study [were] post hoc and therefore not supportive***

of effectiveness." According to the press release, the FDA's RTF letter also stated that PTC's NDA for Translarna "did not contain adequate information regarding the abuse potential of Translarna, a requirement for new molecules that cross the blood-brain barrier."

161. On a February 29, 2016 earnings call with analysts, PTC went on to explain that, based on the RTF letter, the FDA believed PTC's NDA was insufficient because "***relying on the [300-400 meter] subgroup as the main analysis is considered as a post hoc adjustment***" and that the Company's submission therefore "***eliminates data from a majority of enrolled patients***." Following the call, analysts continued to downgrade the stock citing the Company's lack of transparency and eroded credibility. For example, one analyst report entitled "Downgrading As Translarna Becomes a Show-Me Story" stated "[w]e believe there is little transparency on FDA communications and EU pricing proceedings to give investors comfort, ***and importantly, no additional transparency forthcoming despite eroded credibility***. We are downgrading to Market Perform as PTCT has become a show-me story."

162. Another analyst report lowered the price target for the Company and explained:

> More importantly, on the call, PTC provided some color around the RTF letter last week, however several questions remain related to the path forward in the US and additional clinical trial work cannot be ruled out at this point. Despite our earlier move to Neutral [ ], ***we were somewhat surprised by FDA's response regarding the pre-specified subgroup analyses in the ACT DMD study, which will not help growing management credibility concerns.***

ADDITIONAL INDICIA OF SCIENTER

163. As alleged herein, Defendants acted with scienter in that each Defendant: knew or was reckless in not knowing that the public documents and statements issued or disseminated in the name of the Company were materially false and misleading and/or omitted material information that was needed to make the statements made not misleading; knew that such

statements or documents would be issued or disseminated to the investing public; and knowingly and substantially participated or acquiesced in the issuance or dissemination of such statements or documents as primary violations of the federal securities laws.

164. Defendants Peltz and Kovacs (collectively, the "Individual Defendants"), because of their positions with the Company, possessed the power and authority to control the contents of PTC's quarterly reports, press releases, and presentations to securities analysts, money and portfolio managers, and investors, *i.e.*, the market. They were provided with copies of the Company's reports and press releases alleged herein to be misleading prior to or shortly after their issuance and had the ability and opportunity to prevent their issuance or cause them to be corrected. Because of their positions with the Company and their access to material information available to them but not to the public, the Individual Defendants knew that the adverse facts specified herein had not been disclosed to and were being concealed from the public and that the positive representations being made were then materially false and misleading. The Individual Defendants are liable for the false statements pleaded herein.

165. As set forth herein in detail, Defendants, by virtue of their receipt of information reflecting the true facts regarding PTC, their control over, and/or receipt and/or modification of PTC's allegedly materially misleading misstatements and omissions and/or their associations with the Company which made them privy to non-public information concerning PTC, participated in the wrongful scheme alleged herein.

A. Defendants Met with the FDA After PTC's Phase 2b NDA Was Rejected, Receiving Specific Guidance on How to Design Phase 3 for FDA Approval

166. As discussed in Paragraphs 67-75, *supra*, the Company's senior level executives attended meetings with the FDA in 2012 following the rejection of the Company's first NDA, with the specific intent of identifying those issues which had led to the 2011 RTF, ensuring that

those issues were not present in the Phase 3 trial, and otherwise designing Phase 3 to provide stronger evidence of Translarna's efficacy. However, by the end of the Class Period, after the ACT DMD trial failed, Defendants recklessly and inexplicably ceased all communication with the FDA, going against conventional wisdom that orphan drug developers who are granted fast track designation take advantage of their ability to communicate with the FDA "early and often."

167. According to PTC's SEC filings, the Company was in close communication with the FDA early on during the Translarna for nmDMD clinical trials. This is unsurprising in light of the fact that, unlike most drug candidates, "fast track" drug sponsors, are provided with the benefit of having regular communications with the FDA during the clinical trial and NDA submission process. Indeed, PTC met with the FDA (who paid for the 2b trial, in part, through a grant from the Orphan Products Clinical Trials Grant Program) following the completion of the Phase 2b trial and before the Company submitted the 2011 NDA to the FDA. In December 2011, following the FDA's rejection of the NDA for failing to show statistical significance, PTC filed with the FDA a formal dispute resolution request concerning the NDA. The Company also requested review of the issues related to the 2011 RTF and a prospective resubmission of the NDA with updated information and analyses.

168. More importantly, in February 2012, ***PTC discussed the design of a proposed Phase 3 clinical trial with the FDA***. Throughout the Class Period, Defendants would rely on this fact to tout the results of the ACT DMD trial and imply that the FDA supported their conclusion and downplaying analysts concerns that the subgroups and meta-analysis would not support Translarna's approval. For example, in one call with analysts, Peltz noted that the Company even discussed and "agreed upon" the 300-400 meter subgroup and meta-analyses with the FDA during these meetings.

Ritu Baral - Cowen and Company - Analyst

And the -- have you discussed the specific subgroup 300 to 400 meter with FDA at any point during the end of Phase 2 study -- or I'm sorry, end of Phase 2 meetings, or any other type [ERC] meeting?

Stuart Peltz - PTC Therapeutics Inc. – CEO

What we -- obviously, we did this. We built a statistical analysis plan, and put that in, and we -- that was discussed and so, that was part of our plan.

* * *

Joel Beatty - Citigroup – Analyst

Hi, thanks for taking the question. So have you had discussions with the FDA on the degree of consideration they might give to pre-specified meta-analysis? And if so, can you provide any more information on that?

Stuart Peltz - PTC Therapeutics Inc. – CEO

Sure, yes. Thanks for that question. ***The pre-specified meta-analysis was in our statistical analysis plan, which we had discussions with with [sic] the FDA. This was in part, part of the pre-specified plan. So they are well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure. I should make one other point, is that we have had conversations with the FDA. One thing they even said we could say publicly, this is very important for them, and that they are trying to rapidly, giving this a high priority, give the highest priority in order to try and get drugs to these patients.***

169. According to PTC, during that meeting, "***the FDA indicated that the adequacy of data for filing and approval of an NDA would remain review issues***, the FDA had no objections to key elements of [the] proposed trial design."

170. As a result of those meetings the Company knew that the FDA had set forth additional efficacy requirements that the company would be required to meet in order for the FDA to accept the Translarna NDA for review. Because the FDA flatly rejected the 2016 NDA, these efficacy requirements must have been in addition to the results of the Phase 2b trial, the

purported "confirmation" thereof via the 300-400 meter subgroup, and the statistical analyses in the ACT DMD study that Defendants claim supported a showing of efficacy. Indeed, Defendants stated that the FDA approved these subgroups and analyses in their statistical plan and were able to exhibit a statistically significant result in the ACT DMD trial. Had these been the only efficacy requirements set forth by the FTC, the NDA would not have been rejected by the FDA due to a failure to "provide substantial evidence of effectiveness."

171. Alarmingly, after completion of the ACT DMD and prior to the complete submission of the 2016 NDA, the Company did not meet with the FDA to discuss the "adequacy of the data." This is surprising considering that the Company achieved statistically significant results in both the 300-400 subgroup and in the meta-analyses that, according to the Company, were discussed and approved by the FDA.

172. This reckless change in policy can only be explained by the fact that the Company knew, or was reckless in not knowing, that if it met with the FDA and shared the results of the ACT DMD trial ***before submitting the NDA***, the Company would be told that the ACT DMD data was so inadequate on its face that it would not support NDA review, and therefore not to file the NDA. In order to avoid this total loss, PTC instead submitted the NDA without first consulting with FDA, hoping that there might be a chance that FDA would overlook facial inadequacy. Indeed, as analysts described after the FDA issued the RTF letter to Defendants:

> ***We note, again, that PTCT did not meet with FDA after ACT-DMD results were released and before completion of the rolling NDA. The level of miscommunication (or lack of communication) with the agency suggests a major breakdown of the regulatory communication process. It is also at odds with FDA's very public message to orphan drug developers to talk to them "early and often".***
>
> ***Our concern around the lack of quality communication is only deepened by the second point of the RTF: the inadequacy of the abuse potential. We believe this an issue addressed by a large***

> ***proportion of sponsors, and is therefore a relatively straightforward, almost mundane, regulatory requirement that somehow slipped through the cracks of the Translarna filing package.***

173. Had the Defendants met with the FDA prior to NDA submission, as is strongly suggested by the agency, PTC would have known whether or not the NDA contained an omission or inadequacy so severe as to not permit review of the Company's NDA. Therefore, Defendants knew or was deliberately reckless in not knowing: (1) that there was a sever inadequacy in the 2016 NDA and (2) that there was a significant risk that the FDA would issue a RTF letter after receiving the Company's 2016 NDA because of the poor ACT DMD clinical trial results.

174. Accordingly, Defendants knew or were reckless in not knowing that a poor result in the ACT DMD trial would almost certainly result in the FDA refusing to even review the Company's follow-up NDA, and/or that relying primarily on a subgroup and meta-analyses to support a broad label application was facially insufficient and did not demonstrate Translarna's efficacy. In light of these meetings, Defendants also knew and/or were reckless in not knowing that based on the ACT DMD results and lack of communication with the FDA thereafter, there was no reasonable basis to believe that the NDA would be facially adequate to support a substantive review by the FDA.

B. The Rarity With Which the FDA Issues RTF Letters Supports an Implication that Defendants Knew or Recklessly Ignored an Increased Risk of Rejection

175. As discussed in Paragraphs 51-54, *supra*, the FDA's use of a rarely issued RTF letter in response to PTC's 2016 NDA supports a strong inference that Defendants knew the 2016 NDA contained an omission or inadequacy that was so severe as to render the NDA incomplete on its face. From January 2010 to February 2016, out of more than 200 applications, the FDA only issued 18 RTF letters, two of which involved Translarna for nmDMD.

176. By their very nature, RTF letters are reserved for special situations where the deficiency in the NDA is so glaring, that it would be in known to any person who had access to the information in the NDA. At the very least, the issuance RTF letter suggests that the corporate officer responsible for the NDA either knew of the facial deficiency and submitted the NDA anyway, or was reckless in not identifying that the 2016 NDA contained an especially severe omission or inadequacy. Indeed, according to the FDA, "[i]n general, a RTF is based on omissions of ***clearly necessary information…or omissions or inadequacies so severe as to render the application incomplete on its face and where the omissions or inadequacies are obvious, at least once identified, and not a matter of interpretation or judgment about the meaning of data submitted***." RTFs are "***not an appropriate vehicle for dealing with complex issues and close judgments on such matters as balancing risks and benefits, magnitude of clinical effect, acceptability of a plausible surrogate marker, or nuances of study design***."

177. Accordingly, Defendants must have known or were reckless in not knowing that a poor result in the ACT DMD trial would almost certainly result in the FDA refusing to even review the Company's follow-up NDA, and/or that relying primarily on a subgroup and meta-analyses to support a broad label application was facially insufficient and did not demonstrate Translarna's efficacy. The FDA issuance of a RTF letter in response to PTC's 2016 NDA supports a strong inference that Defendants must have known and/or were reckless in not knowing that based on the ACT DMD results and lack of communication with the FDA thereafter, there was no reasonable basis to believe that the NDA would be facially adequate to support a substantive review by the FDA.

C. Defendants' Statements Themselves Support a Strong Inference of Scienter

178. As discussed in Paragraphs 61, 69-70, 75, 78, 84, 90, 92, 95, and 102-103, *supra*, during the Class Period, Defendants spoke at length about: (1) the Company's discussions with

the FDA; (2) the results of the Phase 2b and Phase 3 clinical trials; and (3) the reasons for the FDA's rejection of the two NDAs as discussed in the two RTF letters. For each of these topics of discussion, Defendants were in possession of non-public information that was not disclosed to the investing public. Defendants' scienter is therefore evidenced by their discussion on these topics during the Class Period. Defendants breached their duty under the Federal securities laws by speaking on these topics and failing to fully disclose all relevant material information while doing so.

179. Defendants spoke at length about their conversations with the FDA about the clinical trial design and statistical analysis plan for the ACT DMD study. Defendants were therefore in possession of material non-public information related to their discussions with the FDA, especially as it pertains to any additional efficacy requirements that were disseminated to the Company prior to the ACT DMD results.

180. Similarly, Defendants' detailed knowledge of the Translarna clinical trial results, much of which has still never been released to the public, is evident from the Defendants' in depth discussion of the ACT DMD results. Indeed, many of the professional pharmaceutical analysts who covered the Company during the Class Period and were confused about details of the ACT DMD results relied on Defendant Peltz's knowledge of the clinical results in the wake of the Phase 3 trial. For example, Defendant Peltz's knowledge of the results of poor performing subgroups (*i.e.* the less than 300 meter and greater than 400 meter subgroups baseline) was evident when he was discussing the results of the ACT DMD trial with an analyst from RBC Capital Markets:

Stuart Peltz - PTC Therapeutics Inc. – CEO

> Yes, that's a good question. You have to remember that when we designed the enrollment criteria for ACT DMD, we were targeting

patients in the decline phase with respect to the six minute walk test.

This initially focused our understanding in the patients that was less than 350 meters showed a rapid decline while based on our understanding from the previous phase 2B study. So, subsequently, what we did we redefined the 300 meter to 400 meter subgroup based on the recent MRI data and other data showing that patients under 300 meters were at risk for losing ambulation.

By defining these two key subgroups then, they are clearly corresponding complement subgroups, which are patients with baseline six minute walk distances that are greater than 350 meters, greater than 400 meters, and less than 300 meters.

We think that it's important that from a clinical and scientific reason to think that there are two key sub[groups], that's the less than 350 and the 300 meter to 400 meter subgroup that are the most reliable. So, I think what you can say, is that we those two key subgroups, which then had three other complementary subgroups.

Simos Simeonidis- RBC Capital Markets – Analyst

So there were two subgroups and each one had three, so was it a total of six? Or was it two plus three, a total of five?

Stuart Peltz - PTC Therapeutics Inc. – CEO

The two key subgroups plus the three complement groups, which is five.

181. As of the filing of this Complaint, Defendants have still yet to release or publish the full results of the ACT DMD trial. Indeed, the additional information that was included, yet heavily redacted, in the publication by the British organization NICE, suggests that the data package the Company submitted to regulators following the failed ACT DMD trial was insufficient to support a finding of efficacy. Defendants' knowledge of the NICE information and any other additional information related to the failed results of the ACT DMD trial and not disclosed to the investing public, supports an inference that Defendants acted with scienter when they spoke to the public about the Company's trial results.

182. Defendants were also privy to material non-public information contained in the RTF letters that were sent to the Company following both the 2011 and 2016 NDA process. As discussed above in Paragraphs 65, 96, and 100-103, *supra*, Defendants disclosed to the market that the Company received RTF letters on both occasions, but only disclosed a basic reasoning for why the Company received the letters. Indeed, Defendant Peltz was in possession of such information when he spoke about the reasoning behind the 2016 RTF noting that the Company believes the FDA's reasoning was: "***relying on the [300-400 meter] subgroup as the main analysis is considered as a post hoc adjustment***" and that the Company's submission therefore "***eliminates data from a majority of enrolled patients.***"

183. Accordingly, Defendants must have known or were reckless in not knowing that a poor result in the ACT DMD trial would almost certainly result in the FDA refusing to even review the Company's follow-up NDA, and/or that relying primarily on a subgroup and meta-analyses to support a broad label application was facially insufficient and did not demonstrate Translarna's efficacy. Defendants' statements about the very subject matter that it misled the investing public about support a strong inference that Defendants must have known and/or were reckless in not knowing that based on the ACT DMD results and lack of communication with the FDA thereafter, there was no reasonable basis to believe that the NDA would be facially adequate to support a substantive review by the FDA.

D. Translarna's Importance to the Company Supports a Strong Inference of Scienter Under the Core Operations Doctrine

184. As discussed above in Paragraphs 35-37, *supra*, Translarna was PTC's most important drug. As analysts suggested, "***PTC is all about Translarna, the success or failure of Translarna is likely to define the Company over the next year or so***. . . ." Translarna's potential application to multiple nonsense mutation disorders also meant that the success of a drug as a

whole, depended largely on the success or failure of the ACT DMD trial and subsequent FDA review and approval of the drug. Moreover, Translarna for DMD was the only drug in PTC's stable that was actually generating any product revenue. Indeed, as of February 2016, Translarna for DMD represented 100% of PTC's product revenue. It would be absurd to suggest that Defendants were not aware of details surrounding the sufficiency of the ACT DMD trial results and the subsequent NDA application, filing and approval, including the information provided by the FDA in meetings following the 2011 NDA and 2011 RTF.

185. Indeed, as discussed above in Paragraphs 108-110, 113-114, 118, 121, 124, 126-130, 136-138, 143, 147, and 150-151, both Defendants Peltz and Kovacs were intimately knowledgeable about the results of the Phase 2b and ACT DMD trials and the NDA application to the FDA, as evidenced by their detailed discussions of the clinical trial results on conference calls with analysts. In addition to the trial results, as discussed in Paragraphs 67-70 *supra*, Defendants Peltz and Kovacs also spoke at length about the Company's interactions with the FDA, including right after the 2011 RTF when PTC began designing the ACT DMD trial with assistance from the FDA. Therefore, Defendants, including Defendants Peltz and Kovacs, had access to and concealed information concerning the significant risk that a negative trial result in the ACT DMD trial would result in the FDA refusing to file the NDA.

186. Accordingly, Defendants must have known or were reckless in not knowing that a poor result in the ACT DMD trial would almost certainly result in the FDA refusing to even review the Company's follow-up NDA, and/or that relying primarily on a subgroup and meta-analyses to support a broad label application was facially insufficient and did not demonstrate Translarna's efficacy. Defendants' access to documents and information related to the Company's most critical clinical trial for their most important drug, and the application for

approval of that drug supports a strong inference that Defendants must have known and/or were reckless in not knowing that based on the ACT DMD results and lack of communication with the FDA thereafter, there was no reasonable basis to believe that the NDA would be facially adequate to support a substantive review by the FDA.

E. PTC Was Motivated to Conceal the Likelihood of the FDA Issuing a RTF Letter in Response to the 2016 NDA

187. Defendants were motivated to materially misrepresent to investors the true condition of the Company because their scheme and improper course of conduct: (1) deceived the investing public regarding PTC's business, operations, and management and the intrinsic value of PTC securities; (2) enabled Defendants to artificially inflate the price of PTC securities; and (3) caused Plaintiffs and other members of the Class to purchase PTC securities at artificially inflated prices.

1. Defendants Knew a Failed Result for Translarna for nmDMD Would Put the Entire Translarna Program at Risk

188. Defendants were highly motivated to conceal from the market, the significant risk that that the FDA would refuse to file the 2016 NDA as a result of the severe omissions or deficiencies present in the 2016 NDA. Aside from Translarna for DMD being the Company's flagship product, a RTF from the FDA would likely result in a domino effect of rejections by the FDA for the other orphan drug indications for Translarna including nonsense mutation Cystic Fibrosis and nearly 20 other possible indications that the Company had under development, causing significant and material harm to the Company and its prospects.

2. Defendants Knew That a RTF Letter from the FDA on the 2016 NDA Would Prevent PTC from Generating any Revenues in the US for the Foreseeable Future

189. During the Class Period, Defendants had zero products generating any material revenue in the United States. The approval of Translarna for nmDMD represented the

Company's first opportunity to gain approval for a major drug in the United States and begin generating revenues. While the Company did have other products in development, many of these products were years from FDA approval.

190. Moreover, because orphan drugs command some of the highest prices in the market, Defendants were in a position to earn an enormous profit on each and every prescription of Translarna that was written by a physician. For example, in Europe, PTC was charging close to 300,000 euros per prescription of Translarna for nmDMD. Defendants knew that opportunity would vanish in the important U.S. market in the near-term if the FDA did not review and approve the 2016 NDA for Translarna for DMD.

191. For the same reason, Defendants were motivated to apply for a broad label so that they could profit on all nmDMD patients; even those patients who showed no clinically meaningful benefit in the ACT DMD trial. As discussed above in Paragraph 83, the only subgroup that showed a statistically significant result was the 300-400 meter baseline subgroup. The < 300 and > 400 subgroups demonstrated no clinical benefit as compared to those patients taking placebo. Nonetheless, Defendants submitted an NDA seeking approval for all nmDMD patients. Even those who would not benefit from taking the drug according to the ACT DMD results.

192. During the Class Period, Defendants tried to explain their motivation to seek a label in excess of what the results showed, noting, "if you look at the draft guidelines from the FDA… [L]ook at the very last page. It talks about showing benefit in a subgroup wouldn't necessarily limit labeling." However, this creative logic goes against the plain language in the June 2015 FDA guidance which simply states that subgroups containing a small number of patients can support a broad label, if there is data to support it.

> Labeling Considerations – Efficacy studies that enroll patients across disease stages and phenotypes are encouraged, and ***data from even a relatively small number of patients across different disease subgroups may help to support an indication that includes broader groups of patients***. An indication narrowly restricted to the specific disease state or phenotype enrolled in efficacy trials for dystrophinopathies is unlikely to be approved unless there is specific concern that the demonstrated effect may be limited to that particular group or that the risk is unacceptable in other groups.

3. Defendants Knew an Additional Phase 3 Trial Would be Costly and Time Consuming

193. Defendants were also motivated by the cost and time that the Company would have to expend in order to conduct a follow up clinical trials to support Translarna's efficacy if the FDA determined the data in the completed clinical trials to be inadequate to support approval. Clinical trials are typically costly and often take a year or more to fully enroll, complete and then analyze the data and compile it into an NDA for submission to the FDA. Therefore, PTC was highly motivated to conceal the severe inadequacies contained in the 2016 NDA and the risk that the FDA would refuse to file the 2016 NDA based on the poor results of the ACT DMD "confirmatory" trial.

F. Corporate Scienter Doctrine

194. PTC is liable for the acts of the Individual Defendants and its employees under the doctrine of *respondeat superior* and common law principles of agency, as all the wrongful acts complained of herein were carried out within the scope of their employment with authorization.

195. The scienter of the Individual Defendants and other employees and agents of the Company is imputed to PTC under the doctrine of *respondeat superior* and common law agency principles.

LOSS CAUSATION AND ECONOMIC LOSS

196. The market prices of PTC common stock were artificially inflated by the material misstatements and omissions alleged herein, including those set forth above in Paragraphs 108-110, 113-114, 118, 121, 124, 126-130, 136-138, 143, 147, and 150-151.

197. The artificial inflation in PTC common stock was fully removed after the Company filed the February 23, 2016 Form 8-K with the SEC, which disclosed the materialization of the risk that the FDA would refuse to accept the filing of the Company's NDA for Translarna for nmDMD and that the Company had received a RTF letter from the FDA regarding its NDA for Translarna (for treatment of nmDMD). In the press release, filed as an exhibit to the Company's February 23, 2016 Form 8-K, the Company announced that the RTF Letter stated that PTC's NDA for Translarna "was not sufficiently complete to permit a substantive review."

198. The announcement also revealed the materialization of the risk that the Company would be unable to submit to the FDA for review a substantively complete NDA, a risk which the Company had all but ignored throughout the Class Period. This February 23, 2016 news, as described more fully above, caused the prices of PTC common stock to decline by material and statistically significant amounts, resulting to economic injury to Plaintiffs and other members of the Class.

199. As a result of this news, the price of PTC stock fell from its $28.26 closing price on Monday, February 22, 2016 to $10.84 at the close of trading on Tuesday, February 23, 2016, a loss of $17.42 per share, or 61.6%.

200. As discussed above in Paragraphs 97-98 and 104-106 above, analysts reacted to the news of the Translarna RTF by downgrading the stock and calling into questions the Company's credibility. For example, on February 23, 2016, one analyst report from Anumpam

Rama of J.P. Morgan stated that PTC's shares were "down 40%+ and justifiably so" given the fact that "Currently, there are more questions than answers, including 1) nature / details of RTF, 2) the possibility of additional pivotal trial work in DMD for US approval . . . and 5) credibility of management." Similarly, analysts from RBC Capital Markets explained, "[w]hile there were no details disclosed about the contents of the RTF letter, we view the market sell-off as justified given that 1) [Translarna] failed its confirmatory Phase III trial . . . and 2) FDA has recently shown it will not approve drugs it does not believe work."

PRESUMPTION OF RELIANCE: FRAUD-ON-THE-MARKET

201. At all relevant times, the market for PTC's common stock was an efficient market for the following reasons, among others:

(a) PTC's common stock met the requirements for listing and was listed and actively traded on the NASDAQ during the Class Period, a highly efficient and automated market;

(b) PTC communicated with public investors via established market communication mechanisms, including disseminations of press releases on the national circuits of major newswire services and other wide-ranging public disclosures, such as communications with the financial press and other similar reporting services;

(c) PTC was followed by several securities analysts employed by major brokerage firms who wrote reports that were distributed to the sales force and certain customers of their respective brokerage firms during the Class Period. Each of these reports was publicly available and entered the public marketplace; and

(d) unexpected material news about PTC was reflected in and incorporated into the Company's stock price during the Class Period.

202. As a result of the foregoing, the market for PTC's common stock promptly digested current information regarding PTC from all publicly available sources and reflected such information in PTC's stock price. Under these circumstances, all purchasers of PTC's common stock during the Class Period suffered similar injury through their purchase of PTC's common stock at artificially inflated prices, and a presumption of reliance applies.

203. Alternatively, reliance need not be proven in this action because the action involves omissions and deficient disclosures. Positive proof of reliance is not a prerequisite to recovery pursuant to the ruling of the U.S. Supreme Court in *Affiliated Ute Citizens of Utah v. United States*, 406 U.S. 128 (1972). All that is necessary is that the facts withheld be material in the sense that a reasonable investor might have considered the omitted information important in deciding whether to buy or sell the subject security.

NO SAFE HARBOR; BESPEAKS CAUTION IS NOT APPLICABLE

204. The statutory safe harbor provided for forward-looking statements under certain circumstances does not apply to any of the material misrepresentations and omissions alleged in this Complaint.

205. To the extent certain of the statements alleged to be misleading or inaccurate may be characterized as forward-looking, they were not identified as "forward-looking statements" when made and there were no meaningful cautionary statements identifying important factors that could cause actual results to differ materially from those in the purportedly forward-looking statements.

206. Defendants are also liable for any false or misleading "forward-looking statements" pleaded because, at the time each "forward-looking statement" was made, the

speaker knew the "forward-looking statement" was false or misleading and the "forward-looking statement" was authorized and/or approved by an executive officer of PTC who knew that the "forward-looking statement" was false. Alternatively, none of the historic or present-tense statements made by Defendants were assumptions underlying or relating to any plan, projection, or statement of future economic performance, as they were not stated to be such assumptions underlying or relating to any projection or statement of future economic performance when made, nor were any of the projections or forecasts made by Defendants expressly related to or stated to be dependent on those historic or present-tense statements when made.

CLASS ACTION ALLEGATIONS

207. Plaintiffs bring this action as a class action pursuant to Federal Rule of Civil Procedure 23(a) and (b)(3) individually and on behalf of all other persons and entities that, during the period from November 6, 2014 through February 23, 2016, inclusive, purchased or otherwise acquired the publicly traded common stock of PTC and were damaged thereby. Excluded from the Class are Defendants; present and former officers of PTC; members of PTC's Board of Directors; members of the immediate family (as defined in 17 C.F.R. § 229.404, Instructions (1)(a)(iii) and (1)(b)(ii)) of any such excluded person; the legal representatives, heirs successors, or assigns of any of these individuals and entities; any entities in which Defendants have or had a controlling interest; and any subsidiary or affiliate of PTC; PTC's employee retirement and benefit plan(s) and the participants in such plan(s), and their beneficiaries, to the extent they purchased through such a plan; and the legal representatives, heirs successors, or assigns of any of the foregoing excluded individuals and entities.

208. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, PTC securities were actively traded on the NASDAQ. While the exact number of Class members is unknown to Plaintiffs at this time and

can be ascertained only through appropriate discovery, Plaintiffs believe that there are hundreds or thousands of members in the proposed Class. Record owners and other members of the Class may be identified from records maintained by PTC or its transfer agent and may be notified of the pendency of this action by mail, using the form of notice similar to that customarily used in securities class actions.

209. As of February 24, 2016, the day after the end of the Class Period, there were 34,272,019 shares of the Company's common stock outstanding. Upon information and belief, these shares are held by thousands, if not millions, of individuals located throughout the country and possibly the world. Joinder would be highly impracticable.

210. Plaintiffs' claims are typical of the claims of the members of the Class as all members of the Class are similarly affected by Defendants' wrongful conduct in violation of federal law that is complained of herein.

211. Plaintiffs will fairly and adequately protect the interests of the members of the Class and have retained counsel competent and experienced in class and securities litigation. Plaintiffs have no interests antagonistic to or in conflict with those of the Class.

212. Common questions of law and fact exist as to all members of the Class and predominate over any questions solely affecting individual members of the Class. Among the questions of law and fact common to the Class are:

(a) whether the federal securities laws were violated by Defendants' acts as alleged herein;

(b) whether statements made by Defendants to the investing public during the Class Period misrepresented material facts about the business, operations and management of PTC;

(c) whether Defendants caused PTC to issue false and misleading financial statements during the Class Period;

(d) whether Defendants acted knowingly or recklessly in issuing false and misleading financial statements;

(e) whether the prices of PTC's securities during the Class Period were artificially inflated because of Defendants' conduct complained of herein; and

(f) whether the members of the Class have sustained damages and, if so, what is the proper measure of damages.

213. A class action is superior to all other available methods for the fair and efficient adjudication of this controversy since joinder of all members is impracticable. Furthermore, as the damages suffered by individual Class members may be relatively small, the expense and burden of individual litigation make it impossible for members of the Class to individually redress the wrongs done to them. There will be no difficulty in the management of this action as a class action.

COUNT I

Defendants Violated Section 10(b) of the Securities Exchange Act and SEC Rule 10b-5(b) Promulgated Thereunder

214. Plaintiffs incorporate by reference and reallege each and every allegation above as though fully set forth herein.

215. During the Class Period, Defendants disseminated or approved the false statements specified above, which they knew or deliberately disregarded were misleading in that

they contained misrepresentations and failed to disclose material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading.

216. Defendants violated Section 10(b) of the Securities Exchange Act and Rule 10b-5 promulgated thereunder in that they:

(a) employed devices, schemes, and artifices to defraud;

(b) made untrue statements of material facts or omitted to state material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading; or

(c) engaged in acts, practices, and a course of business that operated as a fraud or deceit upon Plaintiffs and other Class members in connection with their purchases of PTC stock during the Class Period.

217. In addition to the duties of full disclosure imposed on Defendants as a result of their affirmative false and misleading statements to the public, Defendants had a duty to promptly disseminate truthful information with respect to PTC's operations and performance that would be material to investors in compliance with the integrated disclosure provisions of the SEC, including with respect to the Company's revenue and earnings trends, so that the market price of the Company's stock would be based on truthful, complete, and accurate information. SEC Regulations S-X (17 C.F.R. §210.01, *et seq*.) and S-K (17 C.F.R. §229.10, *et seq*.).

218. As a direct and proximate result of Defendants' wrongful conduct, Plaintiffs and the other Class members have suffered damages in connection with their respective purchases and sales of PTC stock during the Class Period, because, in reliance on the integrity of the market, they paid artificially inflated prices for PTC stock and experienced loses when the artificial inflation was released from PTC stock as a result of the revelations and stock price

decline detailed herein. Plaintiffs and the other Class members would not have purchased PTC stock at the prices they paid, or at all, if they had been aware that the market prices had been artificially and falsely inflated by Defendants' misleading statements.

219. By virtue of the foregoing, PTC and the Individual Defendants have each violated Section 10(b) of the Securities Exchange Act, and Rule 10b-5 promulgated thereunder.

COUNT II

The Individual Defendants Violated Section 20(a) of the Securities Exchange Act

220. Plaintiffs incorporate by reference and reallege each and every allegation above as though fully set forth herein.

221. The Individual Defendants acted as controlling persons of PTC within the meaning of Section 20(a) of the Exchange Act. By reason of their controlling positions with the Company, and their ownership of PTC common stock, the Individual Defendants had the power and authority to cause PTC to engage in the wrongful conduct complained of herein, and were culpable participants therein. The Individual Defendants were provided with or had unlimited access to copies of the Company's reports, press releases, public filings, and other statements alleged by Plaintiffs to be misleading before and/or shortly after these statements were issued and had the ability to prevent the issuance of the statements or caused the statements to be corrected. PTC controlled the Individual Defendants and all of its employees. By reason of such conduct, the Individual Defendants are liable pursuant to Section 20(a) of the Exchange Act.

PRAYER FOR RELIEF

WHEREFORE, Plaintiffs demand judgment against Defendants as follows:

A. Determining that the instant action may be maintained as a class action under Rule 23 of the Federal Rules of Civil Procedure, and certifying Plaintiffs as the class representatives;

B. Requiring Defendants to pay damages sustained by Plaintiffs and the Class by reason of the acts and transactions alleged herein;

C. Awarding Plaintiffs and the other members of the Class pre-judgment and post-judgment interest, as well as their reasonable attorneys' fees, expert fees and other costs; and

D. Awarding such other and further relief as this Court may deem just and proper.

DEMAND FOR TRIAL BY JURY

Plaintiffs hereby demand a trial by jury.

Respectfully submitted.

CARELLA, BYRNE, CECCHI, OLSTEIN, BRODY & AGNELLO, P.C.

Dated: January 13, 2017

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